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EPAR summary for the public

Vpriv

velaglucerase alfa

This document is a summary of the European public assessment report (EPAR) for Vpriv. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Vpriv.

What is Vpriv?

Vpriv is a medicine that contains the active substance velaglucerase alfa. It is available as a powder that is made up into a solution for infusion (drip) into a vein.

What is Vpriv used for?

Vpriv is used for the long-term treatment of patients with Gaucher disease. Gaucher disease is a rare inherited disorder, in which people do not have enough of an enzyme called glucocerebrosidase, which normally breaks down a fat called glucocerebroside. Without the enzyme, glucocerebroside builds up in the body, typically in the liver, spleen and bone, which produces the symptoms of the disease: anaemia (low red blood cell counts), tiredness, easy bruising and a tendency to bleed, an enlarged spleen and liver, and bone pain and breaks.

Vpriv is used in patients who have type 1 Gaucher disease, the type that usually affects the liver, spleen and bones.

Because the number of patients with Gaucher disease is low, the disease is considered ‘rare’, and Vpriv was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 9 June 2010.

The medicine can only be obtained with a prescription.

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How is Vpriv used?

Vpriv treatment should be supervised by a doctor experienced in managing patients with Gaucher disease.

The recommended dose of Vpriv is 60 units/kg bodyweight, which is given as a one-hour infusion once every two weeks. The dose can be adjusted according to each patient's symptoms and response to treatment. The first three infusions are given in hospital, but subsequent infusions may be given at home in patients who tolerate the medicine well. Home infusions should be supervised by a healthcare professional trained in emergency measures.

How does Vpriv work?

Gaucher disease occurs because of the lack of an enzyme called glucocerebrosidase. Velaglucerase alfa replaces the missing enzyme in Gaucher disease, helping to break down glucocerebroside and stopping it building up in the body.

How has Vpriv been studied?

In one main study involving 35 patients (including 9 children) with type 1 Gaucher disease, Vpriv was compared with imiglucerase (another medicine for Gaucher disease). The main measure of effectiveness was the improvement in anaemia, one of the symptoms of the disease, after 41 weeks. The study also looked at control of other signs of the disease such as the increase in the number of platelets in the blood, and the reduction of the size of the liver and spleen.

What benefit has Vpriv shown during the studies?

Vpriv was as effective as imiglucerase at reducing anaemia. Vpriv increased the amount of haemoglobin (the protein in red blood cells that carries oxygen) by an average of 1.6 grams per decilitre (from 11.4 g/dl) while imiglucerase increased the amount of haemoglobin by an average of 1.5 g/dl (from 10.6 g/dl). The study also showed that Vpriv is as effective as imiglucerase in controlling other signs of Gaucher disease.

What is the risk associated with Vpriv?

The most common side effects with Vpriv (seen in more than 1 patient in 10) are infusion-related reactions, which include headache, dizziness, hypotension (low blood pressure), hypertension (high blood pressure), nausea (feeling sick), asthenia (weakness) or fatigue (tiredness), and pyrexia (fever) or increased body temperature. The most serious side effects are hypersensitivity (allergic) reactions.

Vpriv must not be used in people who have a severe allergic reaction to velaglucerase alfa or any of the other ingredients.

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

Why has Vpriv been approved?

The CHMP decided that Vpriv's benefits are greater than its risks and recommended that it be given marketing authorisation.

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

The company that markets Vpriv will provide educational material to all doctors and patients expected to use Vpriv, informing them of how to manage the risk of infusion-related reactions when the medicine is given at home.

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

Other information about Vpriv

The European Commission granted a marketing authorisation valid throughout the European Union for Vpriv on 26 August 2010.

The full EPAR for Vpriv can be found on the Agency's website under EMA website/Find medicine/Human medicines/European Public Assessment Reports. For more information about treatment with Vpriv, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Vpriv is available on the Agency's website under EMA website/Find medicine/Human medicines/Rare disease designations.

This summary was last updated in 06-2016.