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EPAR summary for the public

Cerezyme

imiglucerase

This document is a summary of the European public assessment report (EPAR) for Cerezyme. 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 Cerezyme.

What is Cerezyme?

Cerezyme is a powder that is made up into a solution for infusion (a drip into a vein). It contains the active substance imiglucerase.

What is Cerezyme used for?

Cerezyme 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 acid beta-glucosidase, which normally breaks down a fatty waste product called glucosylceramide. Without the enzyme, glucosylceramide builds up in the body, typically in the liver, spleen and bone marrow, 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.

Cerezyme is used in patients who have type 1 Gaucher disease, which does not affect the nerve cells, or type 3 Gaucher disease, which progresses slowly and affects the nerve cells. The patients must have symptoms that are not affecting the nervous system, including one or more of the following conditions:

- anaemia;
- thrombocytopenia (low blood platelet counts);
- bone disease;
- an enlarged liver or spleen.

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The medicine can only be obtained with a prescription.

How is Cerezyme used?

Patients with Gaucher disease should be managed by doctors who are knowledgeable about the disease's treatment. Cerezyme is usually given by infusion every two weeks. The dose and how often the infusions are given need to be adjusted according to each individual patient's symptoms and response to treatment. The first few infusions should be given slowly, but after these, the speed of infusion can be increased under the supervision of a doctor or nurse. After training, the patient or carer can give the infusion at home, if their doctor believes it is appropriate.

How does Cerezyme work?

Gaucher disease has previously been treated using an enzyme called alglucerase, which was prepared from human placentas. Imiglucerase, the active substance in Cerezyme, is a copy of this enzyme, which is produced by a method known as 'recombinant DNA technology': the enzyme is made by a cell that has received a gene (DNA), which enables it to produce the enzyme. Imiglucerase replaces the missing enzyme in Gaucher disease, helping to break down glucosylceramide and stopping it building up in the body.

How has Cerezyme been studied?

For type 1 Gaucher disease, Cerezyme has been studied in three studies involving a total of 40 patients. This is an acceptable number because the disease is rare. The studies compared the ability of Cerezyme and alglucerase to control the symptoms of the disease, such as increasing the number of red blood cells and platelets in the blood, and decreasing the size of the liver and spleen.

For type 3 Gaucher disease, which is extremely rare, the company presented data from published articles and from a special register of Gaucher disease patients.

What benefit has Cerezyme shown during the studies?

The studies have shown that Cerezyme is as safe and effective as alglucerase in controlling the symptoms of Gaucher disease. It has also been shown that patients may safely switch from alglucerase to Cerezyme treatment.

What is the risk associated with Cerezyme?

The most common side effects with Cerezyme (seen in between 1 and 10 patients in 100) are dyspnoea (difficulty breathing), coughing, urticaria (hives) or angioedema (swelling beneath the skin), pruritus (itching), rash and hypersensitivity (allergic) reactions. For the full list of all side effects reported with Cerezyme, see the package leaflet. Patients can develop antibodies (proteins that are produced in response to Cerezyme and can affect treatment) and they should be monitored for any allergic reactions to Cerezyme.

Cerezyme should not be used in people who may be hypersensitive (allergic) to imiglucerase or any of the other ingredients.

Why has Cerezyme been approved?

The CHMP decided that Cerezyme gives effective control of the non-neurological symptoms of types 1 and 3 Gaucher disease. The Committee decided that Cerezyme's benefits are greater than its risks and recommended that it be given marketing authorisation.

Other information about Cerezyme:

The European Commission granted a marketing authorisation valid throughout the European Union for Cerezyme to Genzyme Europe B.V. on 17 November 1997. The marketing authorisation is valid for an unlimited period.

The full EPAR for Cerezyme can be found here. For more information about treatment with Cerezyme, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 08-2010.