

22 November 2012
EMA/756393/2012/rev 3
EMA/H/C/002250

Questions and answers

Refusal of the marketing authorisation for Elelyso (taliglucerase alfa)

On 3 July 2012, the Committee for Medicinal Products for Human Use (CHMP) recommended the refusal of a marketing authorisation for the medicinal product Elelyso, intended for the treatment of type 1 Gaucher disease.

Following the CHMP recommendation, on 25 October 2012, the European Commission adopted a decision refusing the granting of a marketing authorisation, the detailed reasons of which can be found [here](#).

What is Elelyso?

Elelyso is a medicine that contains the active substance taliglucerase alfa. It was to be available as a powder to be made up into a solution for infusion (drip into a vein).

What was Elelyso expected to be used for?

Elelyso was expected to be used for the long-term treatment of patients with type 1 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, bone and some other tissues, causing a wide range of problems such as anaemia (low red blood cell counts), tiredness, easy bruising and a tendency to bleed, an enlarged spleen and liver, and bone pain and fractures.

Because the number of patients with Gaucher disease is low, the disease is considered 'rare', and Elelyso was designated an 'orphan medicine' (a medicine used in rare diseases) on 23 March 2010.

How is Elelyso expected to work?

Elelyso is an 'enzyme replacement therapy'. Enzyme replacement therapy provides patients with the enzyme they are lacking. The active substance in Elelyso, taliglucerase alfa, is a copy of

glucocerebrosidase, which is produced by a method known as 'recombinant DNA technology': it is made by cells outside the human body that have received a gene (DNA) that makes them able to produce it. Taliglucerase alfa replaces the missing enzyme in type I Gaucher disease, helping to break down glucocerebroside, preventing it from building up in the body and causing the symptoms of the disease.

What did the company present to support its application?

The effects of Elelyso were first tested in experimental models before being studied in humans.

A main study involving 33 patients with type 1 Gaucher disease compared the effects of Elelyso given at different doses (30 units/kg versus 60 units/kg). The main measure of effectiveness was the reduction in spleen size after nine months of treatment. The study also looked at liver size, and blood measurements such as haemoglobin and platelet levels.

Why did the CHMP not recommend marketing authorisation?

The CHMP noted that the main study showed that Elelyso led to clinically relevant reductions in the size of the spleen as well as the liver. There were also improvements in haemoglobin levels and blood platelet counts. The side effects seen with Elelyso appear to be similar to those of other enzyme replacement therapies. The CHMP therefore concluded that the benefits of the medicine outweighed its risk in the treatment of type 1 Gaucher disease.

However, the CHMP also concluded that the medicine cannot be granted marketing authorisation in the EU because of the ten-year market exclusivity that had been granted for Vpriv, which was authorised in August 2010 for the same condition. Market exclusivity for orphan medicines is given as an incentive for companies to develop medicines for rare diseases, which may otherwise not be developed due to the high costs and small patient populations. The exclusivity means that another medicine cannot be authorised for the same condition if it is similar to the medicine already authorised. In this case, the CHMP concluded that Elelyso is similar to Vpriv, as they are both enzyme replacement therapies that work in the same way.

The CHMP also considered the legal exemptions that could have allowed Elelyso to be authorised in spite of the Vpriv's market exclusivity. The possible exemptions related to whether Elelyso was clinically superior to Vpriv and whether there were supply problems with Vpriv. However, the CHMP concluded that there is no good evidence that Elelyso would offer patients any important advantages over Vpriv or that Vpriv is in short supply. The Committee therefore recommended the refusal of the marketing authorisation.

More information on medicines for rare diseases, including how they are authorised in EU, can be found [here](#).

What consequences does this refusal have for patients in clinical trials or compassionate use programmes?

The company informed the CHMP that patients receiving the medicine in clinical trials will continue to do so as planned. Patients in compassionate use programmes will also continue to receive the medicine but may be transferred to other treatments at a later date.

If you are in a clinical trial or compassionate use programme with Elelyso and need more information about your treatment, contact the doctor who is giving it to you.

The summary of the opinion on the orphan designation of Elelyso from the Committee for Orphan Medicinal Products for Elelyso can be found on the Agency's website: [ema.europa.eu/Find medicine/Human medicines/Rare disease designation](http://ema.europa.eu/Find/medicine/Human_medicines/Rare_disease_designation).