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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Taliglucerase alfa for the treatment of Gaucher disease

On 23 March 2010, orphan designation (EU/3/10/726) was granted by the European Commission to Protalix B.V., the Netherlands, for taliglucerase alfa for the treatment of Gaucher disease.

The sponsorship was transferred to Pfizer Limited, United Kingdom, in September 2010.

What is Gaucher disease?

Gaucher disease is an inherited disorder that is caused by the lack of an enzyme called glucocerebrosidase. This enzyme normally breaks down a fatty waste product called glucocerebroside. Without the enzyme, glucocerebroside builds up in the body, typically in the liver, spleen and bone marrow. This causes a wide range of symptoms, including anaemia (low red blood cell counts), tiredness, easy bruising and a tendency to bleed, an enlarged spleen and liver, and bone pain and fractures.

Gaucher disease is a long-term, debilitating and life-threatening disease that is associated with a reduced life expectancy if left untreated.

What is the estimated number of patients affected by the condition?

At the time of designation, Gaucher disease affected approximately 0.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 15,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, two medicines, imiglucerase and miglustat, were authorised for the treatment of Gaucher disease in the EU. Imiglucerase is an 'enzyme replacement therapy' that works by replacing the missing enzyme. Miglustat, which blocks the production of glucocerebrosides, can only be used in patients who cannot receive enzyme replacement therapy.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 506,500,000 (Eurostat 2010).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

The sponsor has provided sufficient information to show that taliglucerase alfa might be of significant benefit for patients with Gaucher disease because it may represent an alternative treatment to imiglucerase, should the long-term supply problems that have occurred with this medicine happen again in the future. Taliglucerase alfa may also improve the treatment of patients with the condition, particularly with respect to bone problems. These assumptions will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Taliglucerase alfa is an enzyme replacement therapy that is expected to work by replacing the missing enzyme in Gaucher disease, helping to break down glucocerebroside and stopping it building up in the body. Taliglucerase alfa is produced by a method known as 'recombinant DNA technology': it is made by plant cells that have received a gene (DNA), which make them able to produce the enzyme.

What is the stage of development of this medicine?

The effects of taliglucerase alfa have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with taliglucerase alfa were ongoing in patients with Gaucher disease.

At the time of submission, taliglucerase alfa was not authorised anywhere in the EU for Gaucher disease. Orphan designation of taliglucerase alfa had been granted in the United States of America for Gaucher disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 January 2010 recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Pfizer Limited
Ramsgate Road
Sandwich
Kent CT130NJ
United Kingdom
Telephone: +44 13 04 61 61 61
Telefax: +44 13 04 65 62 21
E-mail: ORPHAN_Enquiries@pfizer.com

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Taliglucerase alfa	Treatment of Gaucher disease
Bulgarian	Талиглюцераза алфа	Лечение на болест на Гоше
Czech	Taligluceráza alfa	Léčba Gaucherovy choroby
Danish	Taliglucerase alfa	Behandling af Gauchers sygdom
Dutch	Taliglucerase alfa	Behandeling van de ziekte van Gaucher
Estonian	Taliglütseraas alfa	Gaucher' tõve ravi
Finnish	Taliglucerase alfa	Gaucherin taudin hoito
French	Taliglucérase alfa	Traitement de la maladie de Gaucher
German	Taliglucerase Alpha	Behandlung der Gaucher-Krankheit
Greek	Ταλιγλουκεράση άλφα	Θεραπευτική αγωγή για την νόσο του Gaucher
Hungarian	Taligluceráz alfa	Gaucher-kór kezelése
Italian	Taliglucerase alfa	Trattamento della malattia di Gaucher
Latvian	Alfa taliglicerāze	Gošē slimības ārstēšana
Lithuanian	Alfa taligliucerazė	Gošė ligos gydymas
Maltese	Taliglucerase alfa	Kura tal-marda ta' Gaucher
Polish	Taligluceraza alfa	Leczenie choroby Gaucher'a
Portuguese	Taliglucerase alfa	Tratamento da doença de Gaucher
Romanian	Taligluceră alfa	Tratamentul bolii Gaucher
Slovak	Taligluceráza alfa	Liečba Gaucherovej choroby
Slovenian	Taligluceraza alfa	Zdravljenje Gaucherove bolezni
Spanish	Taliglucerasa alfa	Tratamiento de la enfermedad de Gaucher
Swedish	Taligluseras alfa	Behandling av Gauchers sjukdom
Norwegian	Taliglucerase alfa	Behandling av Gauchers sykdom
Icelandic	Taliglúcerasi alfa	Meðferð á Gauchers sjúkdómi

[1] At the time of designation