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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Interferon gamma for the treatment of Friedreich's ataxia

On 9 December 2011, orphan designation (EU/3/11/935) was granted by the European Commission to Prof. Roberto Testi, Italy, for interferon gamma for the treatment of Friedreich's ataxia.

What is Friedreich's ataxia?

Friedreich's ataxia is an inherited disease that causes a range of symptoms that worsen over time, including difficulty walking, inability to co-ordinate movements, muscle weakness, speech problems, damage to the heart muscle, and diabetes.

Patients with Friedreich's ataxia do not have enough frataxin, a protein that regulates iron in mitochondria (energy-producing components of cells). This results in a toxic build up of iron within the cells, which in turns results in the production of toxic forms of oxygen that damage cells in the brain, the spinal cord and nerves, as well as in the heart and pancreas.

Friedreich's ataxia is a debilitating and life-threatening disease because of the worsening of symptoms over time. The disease is usually fatal in early adulthood.

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

At the time of designation, Friedreich's ataxia affected less than 0.7 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of 35,000 people, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of Friedreich's ataxia. Different treatments were used to relieve the symptoms of the disease, such as medicines for diabetes and heart problems. Patients were also offered walking aids to allow them to

[*]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 506,300,000 (Eurostat 2011).

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remain as independent as possible, and other devices to assist them with everyday tasks such as eating and taking care of themselves. Speech therapy and physiotherapy were also used.

How is this medicine expected to work?

Interferon gamma is a natural substance produced by the body to help it fight against attacks such as infections caused by viruses. The exact way interferon gamma works in Friedreich's ataxia is not fully understood, but it is thought to increase the production of frataxin, helping to relieve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of interferon gamma have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with interferon gamma in patients with Friedreich's ataxia had been started.

At the time of submission, interferon gamma was not authorised anywhere in the EU for Friedreich's ataxia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2011 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:

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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	Interferon gamma	Treatment of Friedreich's ataxia
Bulgarian	Интерферон гама	Лечение на атаксия на Фридрайх
Czech	Interferon gamma	Léčba Friedrichovy ataxie
Danish	Interferon gamma	Behandling af Friedreichs ataksi
Dutch	Interferon gamma	Behandeling van de ataxie van Friedreich
Estonian	Interferroon gamma	Friedreichi ataksia ravi
Finnish	Interferon gamma	Friedreichin ataksian hoito
French	Interféron gamma	Traitement de l'ataxie de Friedreich
German	Interferon gamma	Therapie der Friedreichschen Ataxie
Greek	Ιντερφερόνη γάμμα	Θεραπεία της αταξίας Friedreich
Hungarian	Interferon gamma	Friedreich ataxia kezelése
Italian	Interferon gamma	Trattamento dell'atassia di Friedreich
Latvian	Gamma interferons	Frīdreiha ataksijas ārstēšana
Lithuanian	Gama interferonas	Fridreicho ataksijos gydymas
Maltese	Interferon gamma	Kura tal-atassja ta' Friedreich
Polish	Interferon gamma	Leczenie ataksji Friedricha
Portuguese	Interferão gamma	Tratamento da ataxia de Friedreich
Romanian	Interferon gamma	Tratamentul ataxiei Friedreich
Slovak	Interferón gamma	Liečba Friedreichovej ataxie
Slovenian	Interferon gama	Zdravljenje Friedreichove atakcije
Spanish	Interferón gamma	Tratamiento de la ataxia de Friedreich
Swedish	Interferon gamma	Behandling av Friedreichs ataxi
Norwegian	Interferon gamma	Behandling av Friedreichs ataksi
Icelandic	Interferón gamma	Meðferð arfgengs mænuslingurs

[1] At the time of designation