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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Interferon gamma for the treatment of idiopathic pulmonary fibrosis

On 29 October 2007, orphan designation (EU/3/07/491) was granted by the European Commission to Foundation for Fatal Rare Diseases, Liechtenstein, for interferon gamma for the treatment of idiopathic pulmonary fibrosis.

The sponsorship was transferred to mondoBIOTECH Laboratories AG, Liechtenstein, in August 2012.

What is idiopathic pulmonary fibrosis?

Fibrosis is the formation of scar tissue that is part of the natural repair process of the body following tissue damage. Idiopathic pulmonary fibrosis consists of a chronic inflammation and progressive formation of fibrous tissue in the lungs. Since the injury causing these changes is unknown, it is called idiopathic. The progressive formation of scars impairs the normal functions of the lung tissue, amongst which is oxygenation of the blood. Symptoms of the condition include persistent cough, progressive severe shortness of breath and recurrent lung infections.

Idiopathic pulmonary fibrosis is a chronically debilitating and life-threatening disease, due to the severe respiratory complications.

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

At the time of designation, idiopathic pulmonary fibrosis affected less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 150,000 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?

No satisfactory methods were authorised at the time of application. Only symptomatic treatments to reduce the inflammation were used (corticosteroids and medicinal products that suppress the immune system); in some patients, lung transplantation may be performed.

[*]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.
At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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How is this medicine expected to work?

Scar formation (fibrosis) is regulated by several substances produced in the body during the inflammation stage that precedes fibrosis in the repair process. Although it is not yet fully understood how interferon gamma acts in idiopathic pulmonary fibrosis, it could stimulate the production of proteins that would prevent fibrosis and re-establish the balance between the molecules that stimulate fibrosis and those that prevent it.

What is the stage of development of this medicine?

The effects of interferon gamma were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with idiopathic pulmonary fibrosis were ongoing.

IFNγ-1b was authorised in the European Union for the treatment of chronic granulomatous disease, at the time of designation.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 September 2007 recommending the granting of this designation.

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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 idiopathic pulmonary fibrosis
Bulgarian	Интерферон гама	Лечение на идиопатична белодробна фиброза
Czech	Interferon gamma	Léčba idiopatické plicní fibrózy
Danish	Interferon gamma	Behandling af idiopatisk lungefibrose
Dutch	Interferon gamma	Behandeling van idiopathische longfibrose
Estonian	Interferroon gamma	Idiopaatilise kopsufibroosi ravi
Finnish	Interferon gamma	Idiopaattisen keuhkofibroosin hoito
French	Interféron gamma	Traitement de la fibrose pulmonaire idiopathique
German	Interferon gamma	Behandlung von Idiopathischer Pulmonaler Fibrose
Greek	Ιντερφερόνη γάμμα	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Interferon gamma	Idiopathiás tüdőfibrózis kezelése
Italian	Interferon gamma	Trattamento della fibrosi polmonare idiopatica
Latvian	Gamma interferons	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	Gama interferonas	Idiopatinės plaučių fibrozės gydymas
Maltese	Interferon gamma	Kura tal-fibrożi pulmonari idjopatika
Polish	Interferon gamma	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Interferão gamma	Tratamento da fibrose pulmonar idiopática
Romanian	Interferon gamma	Tratamentul fibrozei pulmonare idiopatice
Slovak	Interferón gamma	Liečba idiopatickej pľúcnej fibrózy
Slovenian	Interferon gama	Zdravljenje idiopatske pljučne fibroze
Spanish	Interferón gamma	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Interferon gamma	Behandling av idiopatisk lungfibros
Norwegian	Interferon gamma	Behandling av idiopatisk lungefibrose
Icelandic	Interferón gamma	Meðferð sjálfvakinnar bandvefsmyndunar í lungum

[1] At the time of designation