



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF heparin sodium for the treatment of idiopathic pulmonary fibrosis

On 2 September 2004, orphan designation (EU/3/04/223) was granted by the European Commission to Prof Dr W. Seeger, Germany, for heparin sodium for the treatment of idiopathic pulmonary fibrosis.

What is idiopathic pulmonary fibrosis?

Fibrosis is the formation of scar tissue as part of the natural repair process of the body following tissue damage. Idiopathic pulmonary fibrosis consists of a chronic inflammation (a response of the body to the injury caused to the tissue) and progressive formation of fibrous tissue in the walls of the small chambers containing air 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 lung tissue, which are to enable exchange of oxygen and carbon dioxide between air and blood. The symptoms developed are 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 progression of symptoms, severe respiratory complications and short life expectancy.

What are the methods of treatment available?

No satisfactory methods exist that 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) or for some patients lung transplantation was performed.

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

According to the information provided by the sponsor, idiopathic pulmonary fibrosis was considered to affect about 138,000 persons in the European Union.

How is this medicinal product expected to act?

The cause of fibrosis in idiopathic pulmonary fibrosis is unknown. One hypothesis is that one of the mechanisms involved in lung fibrosis is the formation of blood clots in the lungs as a consequence of the inflammation. This would be the starting point of a process that would end in a scar. Theoretically, if the clot formation could be stopped the scar formation might also be reduced or stopped. Heparin is a product that interferes in the complex mechanism of clot formation. The capacity of the blood to make clots is diminished. Administration of the heparin directly into the lung (through inhalation) might have a direct effect on the local clot formation and thus eventually on the reduction of the scar formation.

What is the stage of development of this medicinal product?

The effects of heparin sodium were evaluated in experimental models.

At the time of submission of the application for orphan designation, reports of treatment with the medicinal product in patients with idiopathic pulmonary fibrosis were available.

Heparin sodium was not marketed anywhere worldwide for idiopathic pulmonary fibrosis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 22 July 2004 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

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

For more information:

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*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Heparin sodium	Treatment of idiopathic pulmonary fibrosis
Czech	Heparin sodný	Léčba idiopatické plicní fibrózy
Danish	Heparin natrium	Behandling af idiopatisk lungefibrose
Dutch	Heparine natrium	Behandeling van idiopathische longfibrose
Estonian	Hepariinnaatrium	Idiopaatilise kopsufibroosi ravi
Finnish	Hepariini natrium	Idiopaattisen keuhkofibroosin hoito
French	Héparine sodique	Traitement de la fibrose pulmonaire idiopathique
German	Heparin Natrium	Behandlung von Idiopathischer Pulmonaler Fibrose
Greek	Ηπαρίνη άλας νατρίου	Θεραπεία της Ιδιοπαθούς Πνευμονικής Ίνωσης
Hungarian	Heparin nátrium	Idiopathiás tüdőfibrózis kezelése
Italian	Eparina sodica	Trattamento della fibrosi polmonare idiopatica
Latvian	Heparīna nātrija sāls	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	Heparino natrio druska	Idiopatinės plaučių fibrozės gydymas
Maltese	Heparin sodium	Treatment of idiopathic pulmonary fibrosis
Polish	Sól sodowa heparyny	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Heparina sódica	Tratamento da fibrose pulmonar idiopática
Slovak	Heparín sodný	Liečba idiopatickej pľúcnej fibrózy
Slovenian	Heparin natrij	Zdravljenje idiopatske pljučne fibroze
Spanish	Heparina sódica	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Heparinnatrium	Behandling av idiopatisk lungfibros
Norwegian	Heparinnatrium	Behandling av idiopatisk lungefibrose
Icelandic	Blóðstorkutálmi	Meðferð sjálfvakins netjuherslis í lunga