



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 cystic fibrosis

On 22 May 2006 orphan designation (EU/3/06/371) was granted by the European Commission to Ockham Biotech Limited, United Kingdom, for heparin sodium for the treatment of cystic fibrosis.

What is cystic fibrosis?

Cystic fibrosis is a hereditary (genetic) disease that affects the production of secretions (such as mucus) from the glands in the body. It affects the lungs and the digestive system (gut) in particular. Cystic fibrosis is caused by abnormalities in a gene called 'cystic fibrosis transmembrane conductance regulator' (CFTR). The CFTR gene is responsible for the production of CFTR, a protein that regulates the production of mucus and digestive juices by acting as a chloride ion channel to allow proper movement of salt and water in and out of certain cells in the lungs and other tissues. In patients with cystic fibrosis, there is an overproduction of mucus in the lungs and a reduced production of digestive juices from the pancreas (an organ near the stomach). This leads to long-term infection and inflammation of the lungs and problems with the digestion and absorption of food resulting in poor growth.

Cystic fibrosis is a long lasting and life-threatening disease.

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

At the time of designation cystic fibrosis affected approximately 1.3 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 60,000 people.

What treatments are available?

At the time of submission of the application for orphan drug designation, lung infection and inflammation in cystic fibrosis were mainly treated with physiotherapy and antibiotics. Other medicines used to treat the lung disease included bronchodilators (medicines that help to open up the airways in the lungs) and mucolytics (medicines that help dissolve the mucus in the lungs). In addition, patients are often given other types of medicine such as pancreatic enzymes (substances that help to digest and absorb food) and food supplements. They are also advised to exercise and to undergo physiotherapy.

Heparin sodium might be of potential significant benefit for the treatment of cystic fibrosis because it might improve the long-term outcome of patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

* 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 Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

How is this medicine expected to work?

Heparin sodium is a product mainly known by its ability to dissolve blood clots. The sponsor of this product is developing a formulation of heparin sodium that could be taken by inhalation. In cystic fibrosis, the product is thought to dissolve the mucus and to decrease inflammation. It is expected that using heparin sodium locally in the airways the product might help reducing the symptoms of the disease.

What is the stage of development of this medicine?

The effects of heparin sodium were evaluated in experimental models.

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

Heparin sodium was not authorised anywhere worldwide for treatment of cystic fibrosis, at the time of submission. Orphan designation of heparin sodium was granted in the United Kingdom for treatment of cystic fibrosis.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 5 April 2006 a positive opinion recommending the grant of the above-mentioned 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

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

Language	Active Ingredient	Indication
English	Heparin sodium	Treatment of cystic fibrosis
Czech	Heparin sodný	Léčba cystické fibrózy
Danish	Heparin natrium	Behandling af cystisk fibrose
Dutch	Heparine natrium	Behandeling van cystische fibrose
Estonian	Hepariinnaatrium	Tsüstilise fibroosi ravi
Finnish	Hepariini natrium	Kystisen fibroosin hoito
French	Héparine sodique	Traitement de la mucoviscidose
German	Heparin Natrium	Behandlung zystischer Fibrose
Greek	Ηπαρίνη άλας νατρίου	Θεραπεία της κυστικής ίνωσης
Hungarian	Heparin nátrium	Cisztikus fibrózis kezelése
Italian	Eparina sodica	Trattamento della fibrosi cistica
Latvian	Heparīna nātrija sāls	Cistiskās fibrozes ārstēšana
Lithuanian	Heparino natrio druska	Cistinės fibrozės gydymas
Polish	Sól sodowa heparyny	Leczenie zwłóknienia torbielowatego
Portuguese	Heparina sódica	Tratamento da fibrose quística
Slovak	Heparín sodný	Terapia cystickej fibrózy
Slovenian	Heparin natrij	Zdravljenje cistične fibroze
Spanish	Heparina sódica	Tratamiento de la fibrosis quística
Swedish	Heparinnatrium	Behandling av cystisk fibros
Norwegian	Heparinnatrium	Behandling av cystisk fibrose
Icelandic	Blóðstorkutálmi	Meðferð við slímseigjuskjúkdómi