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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Heparin sodium (inhalation use) for the treatment of cystic fibrosis

First publication	8 August 2006
Rev.1: withdrawal from the Community Register	20 January 2014

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2014 on request of the Sponsor.

On 23 December 2005, orphan designation (EU/3/05/337) was granted by the European Commission to Vectura Group plc, United Kingdom, for heparin sodium (inhalation use) for the treatment of cystic fibrosis.

What is cystic fibrosis?

Cystic fibrosis is a genetic disease. Genes located on structures (the so-called chromosomes) carry the genetic information that determines the characteristics of each individual. In humans, each cell has 23 pairs of chromosomes. For each pair one chromosome is inherited from the mother, and the other from the father. Cystic fibrosis is caused by abnormalities of a specific gene, called CFTR, carried by the seventh pair of chromosomes. Cystic fibrosis appears only when the CFTR gene is abnormal on both chromosomes of the seventh pair. The CFTR gene is responsible for the production of a protein that regulates the outflow of water and salts (like chloride) from cells that cover internal and external surfaces of the body, the so-called epithelial cells. The defective transport of water and salts, due to the lack of the regulatory protein, results in the thickening of the secretions (mucous) in several organs (e.g. lungs, pancreas). This leads to reduced functioning, chronic infection of the lungs and chronic inflammation (a body response to the injury caused to the tissue). In the long run, these events can induce damage to the lung tissue and the disease can become life threatening.

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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 was equivalent to a total of around 61,000 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 submission of the application for the orphan drug designation lung infection and inflammation in cystic fibrosis were treated mainly with antibiotics. These can be taken in a number of ways such as through the mouth, through a vein or they can be inhaled as a fine mist of particles. Other medications to treat the lung disease included bronchodilators, medications that can enlarge the lumen of the airways, and mucolytics, which help to dissolve the lung secretions. Associated treatments included daily exercise and physical therapies and several other types of medications such as pancreatic enzymes and food supplements.

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.

How is this medicine expected to work?

Heparin sodium is a product mainly known by its ability to dissolve blood clots. In addition, the sponsor provided data indicating potential other activities that could be of interest for the treatment of cystic fibrosis patients, such as the ability to break down mucus (mucolytic) and to increase the diameter of the respiratory ways (bronchodilator). The sponsor has developed a formulation for dry powder inhalation to be administered with a commercial device. It is expected that using heparin sodium locally in the airways and having these multiple levels of interactions it might help to reduce the severity of the disease in the long-term.

What is the stage of development of this medicine?

The effects of heparin sodium (inhalation use) were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with cystic fibrosis were initiated.

Heparin sodium (inhalation use) was not authorised anywhere worldwide for treatment of cystic fibrosis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 November 2005 recommending the granting of this designation.

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*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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	Heparin sodium (inhalation use)	Treatment of cystic fibrosis
Czech	Heparin sodný (inhalační podání)	Léčba cystické fibrózy
Danish	Heparin natrium (til inhalation)	Behandling af cystisk fibrose
Dutch	Heparine natrium (inhalatie)	Behandeling van cystische fibrose
Estonian	Hepariinnaatrium (inhalatsioon)	Tsüstilise fibroosi ravi
Finnish	Hepariini natrium (inhalaatioon)	Kystisen fibroosin hoito
French	Héparine sodique (voie inhalée)	Traitement de la mucoviscidose
German	Heparin Natrium (zur Inhalation)	Behandlung zystischer Fibrose
Greek	Ηπαρίνη άλας νατρίου (Χρήση δια ειδπνοής)	Θεραπεία της κυστικής ίνωσης
Hungarian	Heparin nátrium (inhalációs alkalmazás)	Cisztikus fibrózis kezelése
Italian	Eparina sodica (uso inalatorio)	Trattamento della fibrosi cistica
Latvian	Heparīna nātrija sāls (inhalācijām)	Cistiskās fibrozes ārstēšana
Lithuanian	Heparino natrio druska (inhaliuoti)	Cistinės fibrozės gydymas
Polish	Sól sodowa heparyny (podanie wziewne)	Leczenie zwłóknienia torbielowatego
Portuguese	Heparina sódica (via inalatoria)	Tratamento da fibrose quística
Slovak	Heparín sodný (inhalačné použitie)	Terapia cystickej fibrózy
Slovenian	Heparin natrij (za inhaliranje)	Zdravljenje cistične fibroze
Spanish	Heparina sódica (via inhalatoria)	Tratamiento de la fibrosis quística
Swedish	Heparinnatrium (användning för inhalation)	Behandling av cystisk fibros
Norwegian	Heparinnatrium (bruk til inhalasjon)	Behandling av cystisk fibrose
Icelandic	Blóðstorkutálmi (til innöndunar)	Meðferð við slímseigjusjúkdómi

[1] At the time of designation