15 October 2010
EMA/COMP/394803/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Nafamostat mesilate for the treatment of cystic fibrosis

On 20 September 2010, orphan designation (EU/3/10/782) was granted by the European Commission to Mucokinetica Ltd, United Kingdom, for nafamostat mesilate for the treatment of cystic fibrosis.

What is cystic fibrosis?

Cystic fibrosis is a hereditary disease that affects the production of secretions such as mucus in the body. In patients with cystic fibrosis, there is an over-production of mucus in the lungs and a reduced production of digestive juices from the pancreas (an organ near the stomach). This leads to problems with the digestion and absorption of food, resulting in poor growth, and long-term infection and inflammation of the lungs because of excess mucus not being cleared away.

Cystic fibrosis is a long-lasting and life-threatening disease because it severely damages the lung tissue, which leads to problems with breathing and results in shortened life expectancy.

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 equivalent to a total of around 66,000 people, and is below the threshold 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 orphan drug designation, lung infection and inflammation in cystic fibrosis were mainly treated with antibiotics. Other medicines used to treat lung disease included bronchodilators (medicines that help to open up the airways in the lungs) and mucolytics (medicines that help to dissolve the mucus in the lungs). In addition, patients with cystic fibrosis were often given other types of medicines such as pancreatic enzymes (substances that help to digest and absorb food) and food supplements. They were also advised to exercise and to undergo physiotherapy.

[*]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 506,500,000 (Eurostat 2010).

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The sponsor has provided sufficient information to show that nafamostat mesilate might be of significant benefit for patients with cystic fibrosis because it works in a different way to existing treatments and because early studies indicate that it might improve the treatment of patients with this condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

In the airways of patients with cystic fibrosis, the activity of a protein called the epithelial sodium channel (ENaC) is increased. This contributes to the inability of these patients to clear excessive mucus from their airways. Nafamostat mesilate is expected to work by reducing ENaC activity, stimulating the clearance of mucus and relieving the symptoms of the disease. The medicine is expected to be given as an inhaled aerosol.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of nafamostat mesilate in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials with nafamostat mesilate in patients with cystic fibrosis had been started.

At the time of submission, nafamostat mesilate was not authorised anywhere in the EU for cystic fibrosis 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 2 June 2010 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	Nafamostat mesilate	Treatment of cystic fibrosis
Bulgarian	Нафамостат месилат	Лечение на кистозна фиброза
Czech	Nafamostat mesylat	Léčba cystické fibrózy
Danish	Nafamostatmesylat	Behandling af cystisk fibrose
Dutch	Nafamostat mesilaat	Behandeling van cystische fibrose
Estonian	Nafamostatmesülaat	Tsüstilise fibroosi ravi
Finnish	Nafamostatmesilaatti	Kystisen fibroosin hoito
French	Mésylate de nafamostat	Traitement de la mucoviscidose
German	Nafamostatmesilat	Behandlung zystischer Fibrose
Greek	Μεσυλική ναφαμοστάτη	Θεραπεία της κυστικής ίνωσης
Hungarian	Nafamostat-mezilát	Cisztikus fibrózis kezelése
Italian	Nafamostat mesilato	Trattamento della fibrosi cistica
Latvian	Nafamostāta mezilāts	Cistiskās fibrozes ārstēšana
Lithuanian	Nafamostato mesilatas	Cistinės fibrozės gydymas
Maltese	Nafamostat mesilate	Kura tal-fibrożi ċistiku
Polish	Mesylan nafamostatu	Leczenie zwłóknienia torbielowatego
Portuguese	Mesilato de nafamostato	Tratamento da fibrose quística
Romanian	Nafamostat mesilat	Tratamentul fibrozei chistice
Slovak	Nafamostat mezylát	Terapia cystickej fibrózy
Slovenian	Nafamostat mesilat	Zdravljenje cistične fibroze
Spanish	Mesilato de nafamostat	Tratamiento de la fibrosis quística
Swedish	Nafamostatmesilat	Behandling av cystisk fibros
Norwegian	Nafamostatmesilat	Behandling av cystisk fibrose
Icelandic	Nafamóstat mesýlat	Meðferð við slímseigjusjúkdómi

[1] At the time of designation