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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human CXCL8 mutant for the treatment of cystic fibrosis

On 7 June 2013, orphan designation (EU/3/13/1131) was granted by the European Commission to ProtAffin Biotechnologie AG, Austria, for recombinant human CXCL8 mutant for the treatment of cystic fibrosis.

What is cystic fibrosis?

Cystic fibrosis is a hereditary disease that affects the cells in the lungs and the glands in the gut and pancreas that secrete fluids such as mucus and digestive juices. In cystic fibrosis, these fluids become thick and viscous, blocking the airways and the flow of digestive juices. 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 recurrent chest infections and respiratory failure.

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

At the time of designation, cystic fibrosis affected approximately 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 42,000 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 designation, lung infection in cystic fibrosis was mainly treated with antibiotics. Other medicines used to treat the lung disease included anti-inflammatory agents, bronchodilators (medicines that help to open up the airways in the lungs) and mucolytics (medicines that help dissolve the mucus in the lungs). Patients with one form of cystic fibrosis were given the medicine Kalydeco which makes the secretions less thick. In addition, patients with cystic fibrosis were often given other

*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 509,000,000 (Eurostat 2013).

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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.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with cystic fibrosis because it works in a different way to existing treatments and could be used in combination with authorised treatments. Results from experimental studies suggest that it may reduce inflammation in the lungs of patients with cystic fibrosis. 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?

Patients with cystic fibrosis produce increased amounts of a substance called interleukin-8 (IL-8), which attaches to the surface of cells in the lungs, and then attracts neutrophils (a type of white blood cell) and stimulates them to attack the tissue causing inflammation and damage. The medicine is an altered form of IL-8 that attaches to lung cells better than the normal form, but does not stimulate inflammation. By displacing the normal IL-8 in this way, it is expected that the medicine will reduce the inflammation and damage caused by the disease.

The medicine is produced by a method known as 'recombinant DNA technology': it is made by bacteria into which a gene (DNA) has been introduced that makes them able to produce the altered form of IL-8.

What is the stage of development of this medicine?

The effects of recombinant human CXCL8 mutant have been evaluated in experimental models.

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

At the time of submission, the medicine 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 17 April 2013 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	Recombinant human CXCL8 mutant	Treatment of cystic fibrosis
Bulgarian	Рекомбинантен човешки CXCL8 мутант	Лечение на кистозна фиброза
Czech	Rekombinantní lidský CXCL8 mutatovaný	Léčba cystické fibrózy
Danish	Rekombinant human CXCL8 mutant	Behandling af cystisk fibrose
Dutch	Recombinante humane CXCL8-mutant	Behandeling van cystische fibrose
Estonian	Rekombinantne inimese CXCL8 mutant	Tsüstilise fibroosi ravi
Finnish	Yhdistelmä-DNA-tekniikalla valmistettu ihmisen CXCL8-mutantti	Kystisen fibroosin hoito
French	CXCL8 humaine recombinante mutée	Traitement de la mucoviscidose
German	Rekombinante humane CXCL8 Mutante	Behandlung zystischer Fibrose
Greek	Ανασυνδυασμένη ανθρώπινημεταλλαγμένη CXCL8	Θεραπεία της κυστικής ίνωσης
Hungarian	Rekombináns humán CXCL8 mutáns	Cisztikus fibrózis kezelése
Italian	CXCL8 mutante ricombinante umana	Trattamento della fibrosi cistica
Latvian	Cilvēka rekombinants CXCL8 mutants	Cistiskās fibrozes ārstēšana
Lithuanian	Žmogaus rekombinantinis CXCL8 mutantas	Cistinės fibrozės gydymas
Maltese	Mutant taċ-CXCL8 rikombinanti uman	Kura tal-fibrożi ċistiku
Polish	Rekombinowany ludzki CXCL8 mutant	Leczenie zwłóknienia torbielowatego
Portuguese	Mutante de CXCL8 humano recombinante	Tratamento da fibrose quística
Romanian	CXCL8 uman recombinant mutant	Tratamentul fibrozei chistice
Slovak	Rekombinantný ľudský CXCL8 mutant	Terapia cystickej fibrózy
Slovenian	Rekombinantni humani mutant CXCL8	Zdravljenje cistične fibroze
Spanish	CXCL8 mutante recombinante humano	Tratamiento de la fibrosis quística
Swedish	Rekombinant human CXCL8-mutant	Behandling av cystisk fibros
Norwegian	Rekombinant humanCXCL8 mutant	Behandling av cystisk fibrose
Icelandic	Raðbrigða manna CXCL8 stökkbrigði	Meðferð við slímseigjusjúkdómi

[1] At the time of designation