20 December 2011
EMA/COMP/870559/2011
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cysteamine for the treatment of cystic fibrosis

On 9 December 2011, orphan designation (EU/3/11/928) was granted by the European Commission to NovaBiotics Ltd, United Kingdom, for cysteamine 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 problems with breathing and recurrent chest infections.

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

At the time of designation, cystic fibrosis affected approximately 0.7 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 35,000 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). 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 506,300,000 (Eurostat 2011).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with cystic fibrosis it might improve the outcome of patients with this disease, particularly when used in combination with other treatments. 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?

Cysteamine is expected to work by breaking the 'disulfide bonds' (chemical links), which hold the molecules in the mucus together, helping to reduce the thickness of mucus and allowing it to be cleared away more easily.

Cysteamine is also expected to work directly against the bacteria in the lungs. Anti-bacterial activity has been seen with cysteamine in experiments but the exact mechanism is not yet known.

The medicine is to be delivered as an aerosol directly into the patient's airway.

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 cysteamine in experimental models was ongoing.

At the time of submission, no clinical trials with cysteamine in patients with cystic fibrosis had been started.

At the time of submission, cysteamine 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 7 October 2011 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	Cysteamine	Treatment of cystic fibrosis
Bulgarian	Цистеамин	Лечение на кистозна фиброза
Czech	Cysteamin	Léčba cystické fibrózy
Danish	Cysteamin	Behandling af cystisk fibrose
Dutch	Cysteamine	Behandeling van cystische fibrose
Estonian	Tsüsteamiin	Tsüstilise fibroosi ravi
Finnish	Kysteamiini	Kystisen fibroosin hoito
French	Cystéamine	Traitement de la mucoviscidose
German	Cysteamin	Behandlung zystischer Fibrose
Greek	Κυστεαμίνη	Θεραπεία της κυστικής ίνωσης
Hungarian	Ciszteamin	Cisztikus fibrózis kezelése
Italian	Cisteamina	Trattamento della fibrosi cistica
Latvian	Cisteamīns	Cistiskās fibrozes ārstēšana
Lithuanian	Cisteaminas	Cistinės fibrozės gydymas
Maltese	Cysteamine	Kura tal-fibrożi ċistiku
Polish	Cysteamina	Leczenie zwłóknienia torbielowatego
Portuguese	Cisteamina	Tratamento da fibrose quística
Romanian	Cisteamină	Tratamentul fibrozei chistice
Slovak	Cysteamín	Terapia cystickej fibrózy
Slovenian	Cisteamin	Zdravljenje cistične fibroze
Spanish	Cisteamina	Tratamiento de la fibrosis quística
Swedish	Cysteamin	Behandling av cystisk fibros
Norwegian	Cysteamin	Behandling av cystisk fibrose
Icelandic	Sýsteamín	Meðferð við slímseigjusjúkdómi

[1] At the time of designation