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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

L-lysine-N-acetyl-L-cysteinate for the treatment of cystic fibrosis

On 3 January 2001, orphan designation EU/3/01/026 was granted by the European Commission to SMB Technology SA, Belgium, for L-Lysine-N-Acetyl-L-Cysteinate for the treatment of cystic fibrosis.

The sponsorship was transferred to LABORATOIRES SMB SA, Belgium, in February 2011.

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 1.1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 41,000 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).

[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition.

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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.

How is this medicine expected to work?

L-lysine-N-acetyl-L-cysteinate is expected to act as a mucolytic agent; that is it might be able to reduce the viscosity ("thickness" of liquid, resistance of liquid to flow) of mucus, helping the removal of bronchial secretions in patients with cystic fibrosis.

What is the stage of development of this medicine?

The effects of L-lysine-N-acetyl-L-cysteinate were evaluated in experimental models.

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

L-lysine-N-acetyl-L-cysteinate was not marketed anywhere worldwide for cystic fibrosis. Orphan designation of L-lysine-N-acetyl-L-cysteinate was granted in the United States for the management of cystic fibrosis in December 2000.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 December 2000 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	L-Lysine-N-acetyl- L-cysteinate	Treatment of cystic fibrosis
Bulgarian	L- Лизин- N-ацетил-L-цистеинат	Лечение на кистозна фиброза
Czech	L-Lysin-N-acetyl-L-cysteinát	Léčba cystické fibrózy
Danish	L-Lysin-N-Acetylcysteinat	Behandling af cystisk fibrose
Dutch	L-Lysine-N-Acetylcysteïnaat	Behandeling van mucoviscidosis
Estonian	L-Lüsiin-N-atsetüül-L-tsüsteinaat	Tsüstilise fibroosi ravi
Finnish	L-Lysiini-N-asetyylikysteinaatti	kystisenfibroosin hoito
French	N-acétylcystéinate de L-lysine	Traitement de la mucoviscidose
German	L-Lysin-N-Acetylcysteinat	Behandlung der zystischen Fibrose
Greek	L–Λυσίνη-N-Ακετυλοκυστεϊνικό οξύ	θεραπεια της ινοκυστικής νόσου
Hungarian	L-Lizin-N-acetil-L-ciszteinát	Cisztikus fibrózis kezelése
Italian	L-lisina-N-acetilcisteinato	Trattamento della fibrosi cistica
Latvian	L-Lizīna-N-acetil-L-cisteināts	Cistiskās fibrozes ārstēšana
Lithuanian	L-Lizin-N-acetil-L-cisteinatas	Cistinės fibrozės gydymas
Maltese	L-Lysine-N-acetyl-L-cysteinate	Kura tal-fibrożi ċistiku
Polish	N-acetylo-L-cysteinian L-Lizyny	Leczenie zwłóknienia torbielowatego
Portuguese	L-lisina-N-acetilcisteinato	Tratamento da fibrose quística
Romanian	L-lizin-N-acetil-L-cisteinat	Tratamentul fibrozei chistice
Slovak	L-Lyzín-N-acetyl-L-cysteín	Terapia cystickej fibrózy
Slovenian	L-Lizin-N-acetil-L-cisteinat	Zdravljenje cistične fibroze
Spanish	N-Acetil-Cisteinato de L-Lisina	Tratamiento de la fibrosis quística
Swedish	L-Lysin-N-acetylcysteinat	Behandling av cystisk fibros

[1] At the time of transfer of sponsorship