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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phosphatidylinositol and cholesterol for treatment of cystic fibrosis

On 30 August 2011, orphan designation (EU/3/11/896) was granted by the European Commission to Lamellar Biomedical Ltd, United Kingdom, for multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phosphatidylinositol and cholesterol for 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. It mainly affects the lungs and the digestive system (gut). 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 the CFTR protein. This protein regulates the production of mucus and digestive juices by acting as a channel to allow the movement of salt and water in and out of cells in the lungs and other tissues.

In patients with cystic fibrosis, there is the production of thick 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 because it leads to lung tissue damage and infections that result in breathing and heart problems as well as problems with the pancreas resulting in poor absorption of food and diabetes.

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 ceiling for orphan

*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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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 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.

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 early studies indicate that it might improve the outcome of patients with this condition 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?

Patients with cystic fibrosis have been shown to have a reduced amount of 'lamellar bodies' in their lungs. Lamellar bodies are small fat-containing particles that are thought to play important roles in the lung, such as keeping the small airways open, improving the transport of mucus and debris and helping defend against infection and inflammation.

The medicine is expected to work by mimicking the missing lamellar bodies. It is made up of small particles called 'vesicles', containing the same fats found in lamellar bodies and is intended to be delivered to patient's airways with a nebuliser (a special machine that changes the solution into an aerosol that the patient can breathe in).

What is the stage of development of this medicine?

The effects of the medicine 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 10 June 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	Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phospatidylinositol and cholesterol	Treatment of cystic fibrosis
Bulgarian	Мултиламеларна микровезикула, съдържаща фосфатидилхолин, сфингомиелин, фосфатидилетаноламин, фосфатидилсерин, фосфатидилинозитол и холестерол	Лечение на кистозна фиброза
Czech	Multilamelární mikrovezikula obsahující fosfatidylcholin, sfingomyelin, fosfatidyletanolamin, fosfatidylserin, fosfatidylinositol a cholesterol	Léčba cystické fibrózy
Danish	En flerlamellet mikrovesikel indeholdende phosphatidylcholin, sphingomyelin, phosphatidylethanolamin, phosphatidylserin, phosphatidylinositol og cholesterol	Behandling af cystisk fibrose
Dutch	Een multilamellair microvesicle bestaande uit fosfatidylcholine, sfingomyeline, fosfatidylethanolamine, fosfatidylserine, fosfatidylinositol en cholesterol	Behandeling van cystische fibrose
Estonian	Multilamellaarne mikrovesiikel, mis sisaldab fosfatidüülkoliini, sfingomüeliini, fosfatidüül-etanoolamiini, fosfatidüülseriini, fosfatidüül-inositooli ja kolesterooli	Tsüstilise fibroosi ravi
Finnish	Fosfatidylkoliinia, sfingomyeliinia, fosfatidyletanolamiinia, fosfatidylseriinia ja fosfatidylinositolia ja kolesterolia sisältävä multilamellaarinen mikrovesikkeli	Kystisen fibroosin hoito
French	Microvésicule multilamellaire, composée de phosphatidylcholine, sphingomyéline, phosphatidyléthanolamine, phosphatidylsérine, phosphatidylinositol et de cholesterol	Traitement de la mucoviscidose
German	Multilamellares Mikrovesikel bestehend aus Phosphatidylcholin, Sphingomyelin, Phosphatidylethanolamin, Phosphatidylserin, Phosphatidylinositol und Cholesterin	Behandlung zystischer Fibrose
Greek	Πολυφυλλώδες μικροκυστίδιο αποτελούμενο από φωσφατιδυλοχολίνη, σφιγγομυελίνη, φωσφατιδυλοαιθανολαμίνη, φωσφατιδυλοσερίνη, φωσφατιδυλοϊνοσιτόλη και χοληστερόλη	Θεραπεία της κυστικής ίνωσης
Hungarian	Foszfatidilkolinból, szfingomielinből, foszfatidiletanolaminból, foszfatidilszerinből, foszfatidilinozitolból és koleszterinből álló multilamelláris, mikroszkópikus méretű hólyag	Cisztikus fibrózis kezelése

[1] At the time of designation

Language	Active ingredient	Indication
Italian	Microvescicole multilamellari comprendente fosfatidilcolina, sfingomielina, fosfatidiletanolamina, fosfatidilserina, fosfatidilinositolo e colesterolo.	Trattamento della fibrosi cistica
Latvian	Daudzslāņu mikropūslītis, kurā atrodas fosfatidilholīns, sfingomielīns, fosfatidiletanolamīns, fosfatidilserīns, fosfatidilinositols un holesterīns	Cistiskās fibrozes ārstēšana
Lithuanian	Daugiasluoksnė mikropūslelė sudaryta iš fosfatidilcholino, sfingomielino, fosfatidiletanolamino, fosfatidilserino, fosfatidilinozitolio ir cholesterolio	Cistinės fibrozės gydymas
Maltese	Mikrobużżieqa multilamellari magħmuls minn phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phosphatidylinositol u kolesterol	Kura tal-fibrożi ċistiku
Polish	Wielowarstwowy mikropęcherzyk zawierający fosfatydylocholinę, sfingomielinę, fosfatydyloetanolaminę, fosfatydyloserynę, fosfatydyloinozytol i cholesterol	Leczenie zwłóknienia torbielowatego
Portuguese	Microvesícula multilamelar composta por fosfatidilcolina, esfingomielinas, fosfatidiletanolamina, fosfatidilserina, fosfatidilinositol e colesterol	Tratamento da fibrose quística
Romanian	Microveziculă multi-lamelară care are în compoziţia sa fosfatidilcolină, sfingomielină, fosfatidiletanolamină, fosfatidil serină, fosfatidil inozitol şi colesterol	Tratamentul fibrozei chistice
Slovak	Multilamelárna mikrovezikula obsahujúca fosfatidylcholín, sfingomyelín, fosfatidyletanolamín, fosfatidylserín, fosfatidylonozidol a cholesterol	Terapia cystickej fibrózy
Slovenian	Multilamelarni mikrovezikel, ki vsebuje fosfatidikolin, sfingomielin, fosfatidiletanolamin, fosfatidilserin, fosfatidilinositol in holesterol	Zdravljenje cistične fibroze
Spanish	Microvesícula multilamelar que comtiene fosfatidilcolina, esfingomielina, fosfatidiletanolamina, fosfatidilserina, fosfatidilinositol y colesterol	Tratamiento de la fibrosis quística
Swedish	Multilamellär mikrovesikel som består av fosfatidylkolin, sfingomyelin, fosfatidyletanolamin, fosfatidylserin, fosfatidylinositol och kolesterol	Behandling av cystisk fibros
Norwegian	Multilamellær mikrovesikkel som består av fosfatidylkolin, sfingomyelin, fosfatidyletanolamin, fosfatidylserin, fosfatidylinositol og kolesterol	Behandling av cystisk fibrose
Icelandic	Fjöllaga örblaðra sem inniheldur fosfatidýlkólín, sphingómýelín, fosfatidýletanólamín, fosfatidýlserín, fosfatidýlínósítól og kólesteról	Meðferð við slímseigjusjúkdómi