



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid for the treatment of cystic fibrosis

On 27 October 2011, orphan designation (EU/3/11/927) was granted by the European Commission to Pharm Research Associates (UK) Limited for sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid 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 leading to problems with the digestion and absorption of food. This results in poor growth, recurrent and chronic 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 1.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 61,000 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 submission of the application for orphan designation, lung infection and inflammation in cystic fibrosis were mainly treated with antibiotics. Other medicines used to treat the lung disease included bronchodilators (medicines that help to open up the airways in the lungs), corticosteroids and

*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).



mucoytics (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 sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid might be of significant benefit for patients with cystic fibrosis because early studies show that it works in a different way to existing treatments and it might improve the treatment of patients, particularly if used in combination with existing methods. 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 patients with cystic fibrosis, mucus sticks to the surfaces of the airways, making it difficult for the airways to remain open. This medicine is a surfactant. Surfactants are substances that lower the surface tension of a liquid, making it less viscous and reducing its ability to stick to surfaces. Like the naturally occurring surfactant in human lungs called 'human pulmonary surfactant protein B', the medicine is expected to have a 'detergent-like' effect on the mucus, preventing it from sticking to the airway surfaces. This is expected to lead to easier removal of the mucus from the lungs and improvement in lung function and breathing.

What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicinal product in patients with cystic fibrosis had started.

At the time of submission, the medicinal product was not authorised anywhere in the EU for cystic fibrosis. Orphan designation of the medicinal product had been granted in the United States for cystic fibrosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 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

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid	Treatment of cystic fibrosis
Bulgarian	Синапултид, дипалмитоилфосфатидилхолин, палмитоил-олеил фосфатидилглицерол, натриева сол и палмитова киселина	Лечение на кистозна фиброза
Czech	Sinapultid, dipalmitoylfosfatidylcholin, palmitoyl-oleoyl fosfatidylglycerol, chlorid sodný a kyselina palmitová	Léčba cystické fibrózy
Danish	Sinapultid, dipalmitoylfosfatidylcholin, palmitoyl-oleoyl fosfatidylglycerol, natriumsalt og palmitinsyre	Behandling af cystisk fibrose
Dutch	Sinapultide, dipalmitoyl-fosfatidylcholine, palmitoyl-oleoyl-fosfatidylglycerol, natriumzout en palmitinezuur	Behandeling van cystische fibrose
Estonian	Sinapultiid, dipalmitoüülfosfatidüülkoliin, palmitoüül-oleüül fosfatidüülgütserool, naatriumsool ja palmitiinhape	Tsüstilise fibroosi ravi
Finnish	Sinapultidi, dipalmitooylifosfatidyylikoliini, palmitooyli-oleyllifosfatidyyliglyseroli, natriumsuola ja palmitiinihappo	Kystisen fibroosin hoito
French	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoylphosphatidylglycerol, sel de sodium et acide palmitique	Traitement de la mucoviscidose
German	Sinapultid, Dipalmitoylphosphatidylcholin, Palmitoyl-oleoylphosphatidylglycerol, Natriumsalz und Palmitinsäure	Behandlung zystischer Fibrose
Greek	Σιναπουλτιδη, διπαλμιτοϋλοφωσφατιδυλοχολινη, παλμιτοϋλικη-ελαιουλικη φωσφατιδυλογλυκερολη και παλμιτικό οξύ	Θεραπεία της κυστικής ίνωσης
Hungarian	Sinapultid, dipalmitoöl-foszfátidil-kolin, palmitoöl-oleoöl-foszfátidil-glicerin, nátriumsó és palmitinsav	Cisztikus fibrózis kezelése
Italian	Sinapultide, dipalmitoölfosfatidilcolina, palmitoöl-oleoöl-fosfatidilglicerolo, sale sodico e acido palmitico	Trattamento della fibrosi cistica
Latvian	Sinapultīds, dipalmitoīlfosfatidilholīns, palmitoīl-oleoīl-fosfatidilglicerīns, nātrija sāls un palmitīnskābe	Cistiskās fibrozēs ārstēšana
Lithuanian	Sinapultidas, dipalmitoīlfosfatidilcholinas, palmitoīl-oleoīl fosfatidilglicerolis, natrio druska ir palmitino rūgštis	Cistinės fibrozės gydymas

¹ At the time of designation

Language	Active ingredient	Indication
Maltese	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol, melh tas-sodju, u palmitic acid	Kura tal-fibrozi čistiku
Polish	Synapultyd, sól sodowa dipalmitoilofosfatydylocholine, sól sodowa palmitoil-oleoilofosfatydyloglicerolu, i kwas palmitynowy	Leczenie zwłóknienia torbielowatego
Portuguese	Sinapultide, dipalmitoilfosfatidilcolina, palmitoiloleoilfosfatidilglicerol, sal de sódio e ácido palmítico	Tratamento da fibrose quística
Romanian	Sinapultid, dipalmitoil-fosfatidilcolină, palmitoil-oleoil fosfatidilglicerol, sare sodică și acid palmitic	Tratamentul fibrozei chistice
Slovak	Sinapultid, dipalmitoylfosfatidylcholín, palmitoyl-oleoyl fosfatidylglycerol, sodná soľ a kyselina palmitová	Terapia cystickej fibrózy
Slovenian	Sinapultid, dipalmitoil fosfatidilholin, palmitoil-oleoil fosfatidilglicerol, natrijeva sol in palmitinska kislina	Zdravljenje cistične fibroze
Spanish	Sinapultida, dipalmitoilfosfatidilcolina, palmitoiloleoilfosfatidilglicerol, sal sódica y ácido palmítico	Tratamiento de la fibrosis quística
Swedish	Sinapultid, dipalmitylfosfatidylkolin, palmityloleoylfosfatidylglycerol, natriumsalt och palmitinsyra	Behandling av cystisk fibros
Norwegian	Sinapultid, dipalmitoylfosfatidylkolin, palmitoyl-oleoyl fosfatidylglyserol, natriumsalt og palmitinsyre	Behandling av cystisk fibrose
Icelandic	Sinapultide, dipalmitoyllesipín, palmitoyl-oleoyl fosfatidylglýseról, natríumsalt og palmitínsýra	Meðferð við slímseigjusjúkdómi