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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol and palmitic acid for the treatment of acute lung injury

On 4 February 2002, orphan designation (EU/3/01/079) was granted by the European Commission to Discovery Laboratories, Inc., United Kingdom, for sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol and palmitic acid (Surfaxin) for the treatment of acute lung injury.

The sponsorship was transferred Pharm Research Associates (UK) Limited, United Kingdom, in September 2010.

What is acute lung injury?

Acute lung injury (ALI) is a pulmonary disorder characterised by lesions of lung cells and acute lung inflammation. It may occur as a result of direct injury to the lung such as toxic gas inhalation or chest trauma, or it may be associated with a wide variety of disorders affecting other organs such as blood infections, non-thoracic trauma, acute pancreatitis or cardio-vascular shock. ALI is a life-threatening condition.

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

At the time of designation, acute lung injury affected approximately 1.8 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 68,000 people, and is below the threshold 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). This estimate is based on available information and calculations presented by the sponsor at the time of the application and may thus differ from the true number of patients affected by the condition.



What treatments are available?

There was no medicinal product specifically authorised to treat this condition in the European Union at the time of submission of the application for orphan drug designation. Beside correction of the cause of ALI when possible, available therapies are supportive, consisting in symptomatic measures (including oxygen and in severe cases mechanical ventilation).

How is this medicine expected to work?

Surfaxin is a surfactant agent. Surfactant substances are naturally produced in lungs and contribute to maintain pulmonary alveoli open and thus functional. The natural surfactant is destroyed in patients with ALI. Surfaxin is intended to compensate the loss of natural surfactant in patients with ALI and thus aims to improve the pulmonary function of these patients.

What is the stage of development of this medicine?

The effects of Surfaxin have been evaluated in experimental models and clinical investigations to measure the effect of Surfaxin in ALI were ongoing at the time of submission of the application for orphan designation. Patients included are adults presenting the most severe form of ALI: acute respiratory distress syndrome.

Surfaxin had not been authorised in any country at the time of submission of the application for orphan designation. In the US orphan drug status was granted in the treatment of acute respiratory distress syndrome in adults.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 November 2001 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol and palmitic acid	Treatment of acute lung injury
Bulgarian	Синапултид, дипалмитоилфосфатидилхолин, палмитоил-олеил фосфатидилглицерол и палмитова киселина	Лечение на остра белодробна недостатъчност
Czech	Sinapultid, dipalmitoylfosfatidylcholin, Palmitoyl-oleoyl fosfatidylglycerol a kyselina palmitová	Léčba akutního poškození plic
Danish	Sinapultid, dipalmitoylfosfatidylcholin, palmitoyl-oleoyl phosphatidylglycerol og palmitinsyre	Behandling af akut lungeskade
Dutch	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol en palmitinezuur	Behandeling van Acute Longbeschadiging
Estonian	Sinapultide, Dipalmitoüülfosfatidüülkoliin, Palmitoüüloleoüülfostatidüülgütserool ja Palmitiinhape	Ägeda kopsuvigastuse ravi
Finnish	Sinapultiidi, Dipalmitolifosfatidylkoliini, palmitolioleoli fosfatidylglyseroli ja palmitiinihappo	Akuutin keuhkovamman hoito
French	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoylphosphatidylglycerol et acide palmitique	Traitement de l'agression pulmonaire aiguë
German	Sinapultide, Dipalmitoylphosphatidylcholin, Palmitoyl-oleoylphosphatidylglycerol und Palmitinsäure	Behandlung des akuten Lungenversagens
Greek	Σιναπουλτιδη, διοφωσφατιδυλοχολίνη, παλμιτοϋλική-ελαιοϋλική φωσφατιδυλογλυκερόλη και παλμιτικό οξύ	Θεραπεία της οξείας πνευμονικής βλάβης
Hungarian	Sinapultide, Dipalmitoil-foszfátidil-kolin, Palmitoil-oleoil-foszfátidil-glicerín és palmitinsav	Akut tüdőkárosodás kezelése
Italian	Sinapultide, dipalmitoilfosfatidilcolina, palmitoiloleoil fosfatidilglicerolo e acido palmitico	Trattamento della Lesione polmonare acuta
Latvian	Sinapultīds, dipalmitoilfosfatidilholīns, palmitoil-oleoil-fosfatidilglicerīns un palmitīnskābe	Akūta plaušu bojājuma ārstēšana

¹ At the time of transfer of sponsorship

Language	Active ingredient	Indication
Lithuanian	Sinapultidas, dipalmitoilfosfatidilcholinas, palmitoiloleoilfosfatidilglicerolis, ir palmitino rūgštis	Ūmaus plaučių pažeidimo gydymas
Maltese	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleoyl phosphatidylglycerol u palmitic acid	Kura ta' korriment akut fil-pulmun
Polish	Synapultyd, dipalmitoilofosfatydylocholina, palmitoiloleoilofosfatydyloglicerol i kwas palmitynowy	Leczenie ostrego uszkodzenia płuc
Portuguese	Sinapultide, dipalmitoilfosfatidilcolina, palmitoiloleoilfosfatidilglicerol e ácido palmítico	Tratamento da lesão pulmonar aguda
Romanian	Sinapultidă, dipalmitoilfosfatidilcolină, palmitoil-oleoil fosfatidilglicerol și acid palmitic	Tratamentul leziunilor pulmonare acute
Slovak	Sinapultid, dipalmitoylfosfatidylcholín, palmitoyl-oleoyl fosfatidylglycerol a kyselina palmitová	Liečba akútneho poškodenia pľúc
Slovenian	Sinapultid, palmitoil-oleoil fosfatidilglicerol, dipalmitoil fosfatidilholin, palmitinska kislina	Zdravljenje akutne poškodbe pljuč
Spanish	Sinapultida, dipalmitoilfosfatidilcolina, palmitoiloleoilfosfatidilglicerol y ácido palmítico	Tratamiento de la lesión pulmonar aguda
Swedish	Sinapultid, Dipalmitylfosfatidylkolin, palmityl-oleoyl fosfatidylkolin och palmitinsyra	Behandling av akut lungskada