



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in August 2004 on request of the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF porcine lung surfactant for treatment of acute lung injury

On 19 September 2001, orphan designation (EU/3/01/057) was granted by the European Commission to Leo Pharmaceutical Products, Denmark, for porcine lung surfactant for treatment of acute lung injury.

What is acute lung injury?

Tiny air sacs called alveoli are located at the tips of the lungs. The alveoli are responsible for exchanging oxygen and carbon dioxide between air and blood. When an infection or a disease injures the lungs, blood and fluid begin to leak into the alveoli. When this happens, air cannot enter the alveoli, which means that the normal functions of the lung tissue are impaired. This will lead to inflammation (a response of the body to the injury caused to the tissue) and progressive formation of scar tissue in the walls of the alveoli. The patient will develop an increasing shortness of breath. There are many possible causes of acute lung injury such as inhaling high concentrations of smoke, toxins, or oxygen; severe burns; blood infection; lung infection; or trauma to other parts of the body. Acute lung injury is a life-threatening condition.

What are the methods of treatment available?

No medicinal products were authorised for the treatment of acute respiratory distress syndrome in the Community at the time of submission of the application for orphan drug designation. The treatment options for acute lung injury were limited to symptomatic care like ventilator support. Antibiotics were also used to treat the infections and reduce the inflammation.

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

According to the information provided by the sponsor, acute lung injury was considered to affect about 67,860 persons in the European Union.

How is this medicinal product expected to act?

Lung surfactant is a molecule formed by the association of protein and fat that coats the alveoli of the lung. It keeps the alveoli open, thus ensuring a stable surface for oxygen passage. Porcine lung surfactant is a natural lung surfactant extracted from pig lungs that might support and improve the lung function.

What is the stage of development of this medicinal product?

The effects of porcine lung surfactant were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with acute lung injury were ongoing.

Porcine lung surfactant was not marketed anywhere worldwide for treatment of acute lung injury or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 18 July 2001 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

Sponsor's contact details:

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Patients' association contact point: Not available

*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. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Translations of the active ingredient and indication in all EU languages

LANGUAGE	Active Ingredient	Indication
English	Porcine lung surfactant	Treatment of acute lung injury
Danish	Lungesurfaktant fra svin	Behandling af akut lungeskade
Dutch	Surfactant uit varkenslongen	Behandeling van Acute Longbeschadiging
Finnish	Sian keuhkosurfaktantti	Akuutin keuhkovamman hoito
French	Surfactant pulmonaire d'origine porcine	Traitement de l'agression pulmonaire aiguë
German	Porcines Lungen Surfactant	Behandlung des akuten Lungenversagens
Greek	Επιφανειοδραστικός παράγων χοίρειων πνευμόνων	Θεραπεία της οξείας πνευμονικής βλάβης
Italian	Surfattante polmonare porcino	Trattamento della Lesione polmonare acuta
Portuguese	Surfactante pulmonar porcino	Tratamento da lesão pulmonar aguda
Spanish	Surfactante pulmonar porcino	Tratamiento de la lesión pulmonar aguda
Swedish	Porcin lungsurfaktant	Behandling av akut lungskada