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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2009 on request of the sponsor.

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of lusupultide for the treatment of acute respiratory distress syndrome

On 17 January 2001, orphan designation (EU/3/00/016) was granted by the European Commission to Byk Gulden Lomberg Chemische Fabrick GmbH, Germany, for lusupultide for the treatment of acute respiratory distress syndrome.
The sponsor Gulden Lomberg Chemische Fabrik GmbH changed name to ALTANA Pharma AG in September 2002.
The sponsorship was transferred to Nycomed GmbH, Germany in October 2007.

What is acute respiratory distress syndrome?
Alveoli are tiny air sacs in the lungs where the oxygen is passed into the blood. When the lungs are injured by infection or disease, blood and fluid begin to leak into the alveoli. When this happens, oxygen cannot enter the alveoli, which means oxygen is no longer getting into the blood. Because the lungs are inflamed and filled with fluid, the patient finds it increasingly difficult to breathe. The inflammation in the lungs leads to scarring. The lungs eventually become stiff with scar tissue and breathing becomes very difficult. There are many possible causes of the type of lung injury that leads to acute respiratory distress syndrome. These include inhaling high concentrations of smoke, toxins, or oxygen; severe burns; blood infection; pneumonia (inflammation in the lungs); pancreatitis (inflammation of the pancreas, an organ close to the stomach) or trauma to other parts of the body. Acute respiratory distress syndrome is life-threatening.

What is the estimated number of patients affected by the condition?
At the time of designation respiratory distress syndrome affected 0.5 to 2.3 in 10,000 persons in 10,000 people in the European Union (EU) [*]. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 19,000 to 87,000 people.

What treatments are 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 a majority of acute respiratory distress syndrome patients were limited to symptomatic care like oxygen treatment. Antibiotics and steroids may also be used to treat infections and reduce inflammation.

[*] 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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How is this medicine expected to work?
Lung surfactant is a protein and fat complex that coats the alveoli of the lung. It reduces surface tension and keeps the alveoli open, thus ensuring a stable surface for oxygen passage. Lusupultide is a human-like lung surfactant and, according to the sponsor, lusupultide ultimately will help the patient to breath better.

What is the stage of development of this medicine?
The effects of lusupultide were evaluated in experimental models. At the time of submission of the application for orphan designation, two clinical trials in patients with acute respiratory distress syndrome were completed.

Lusupultide was not marketed anywhere worldwide for the treatment of acute respiratory distress syndrome, at the time of submission. Orphan designation of lusupultide was granted in the United States for the treatment of adult respiratory distress syndrome

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 21 November 2000 a positive opinion recommending the grant of the above-mentioned 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:
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Patients' association contact point: Not available

Translations of the active ingredient and indication in all official EU languages.

Language	Active Ingredient	Indication
English	Lusupultide	Treatment of Acute Respiratory Distress Syndrome
Bulgarian	Лусупултид	Лечение на остър респираторен дистрес синдром
Czech	Lusupultid	Léčba syndromu akutní dechové tísně
Danish	Lusupultid	Behandling af akut respiratorisk distress syndrom
Dutch	Lusupultide	Behandeling van Acute Respiratory Distress Syndrome
Estonian	Lusupultiid	Ägeda respiratoorse distressi sündroomi ravi
Finnish	Lusupultidi	Akuutin hengitysvaikeusoireyhtymän hoito
French	Lusupultide	Traitement du Syndrome de Détresse Respiratoire Aiguë
German	Lusupultide	Behandlung des Akuten Atemnotsyndroms
Greek	Lusupultide	Θεραπευτική αγωγή για το σύνδρομο οξείας αναπνευστικής δυσχέρειας
Hungarian	Lusupultid	Akut respiratorikus distressz szindróma kezelése
Italian	Lusupultide	Trattamento della sindrome da sofferenza respiratoria acuta
Latvian	Lusupultīds	Akūta respiratorā distresa sindroma ārstēšana
Lithuanian	Lusupultidas	Ūminio kvėpavimo sutrikimo sindromo gydymas
Maltese	Lusupultide	Kura ta’ sindrome ta’ tbatija respiratorja akuta
Polish	Lusupultide	Leczenie zespołu ostrej niewydolności oddechowej
Portuguese	Lusupultide	Tratamento da Síndrome de Deficiência Respiratória Aguda
Romanian	Lusupultidă	Tratamentul sindromului de detresă respiratorie acută
Slovak	Lusupultid	Liečba syndrómu akútnej respiračnej tiesne
Slovenian	Lusupultid	Zdravljenje sindroma akutne dihalne stiske
Spanish	Lusupultida	Tratamiento del síndrome de insuficiencia respiratoria aguda
Swedish	Lusupultid	Behandling av akut respiratoriskt distress-syndrom