



*Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2009 on request of the Sponsor.*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 aspiration pneumonitis requiring intubation and mechanical ventilation**

On 10 July 2007, orphan designation (EU/3/07/457) was granted by the European Commission to ALTANA Pharma AG, Germany, for lusupultide for the treatment of aspiration pneumonitis requiring intubation and mechanical ventilation.

The sponsorship was transferred to Nycomed GmbH, Germany, in September 2007.

#### **What is aspiration pneumonitis requiring intubation and mechanical ventilation?**

Aspiration pneumonitis requiring intubation and mechanical ventilation results from chemical damage to the lungs, caused by regurgitation of content from the stomach. Lung damage due to aspiration of stomach (gastric) contents occurs in two phases: first there is a chemical burn from the natural acid secretions, which immediately injures the lining of the airways to the periphery; then a secondary acute inflammatory response occurs, 2 to 3 hours later. In severe cases, patients develop acute respiratory insufficiency and require assisted ventilation through a tube in the trachea, which is connected to an automatic ventilator machine.

The condition is life-threatening, due to acute respiratory insufficiency.

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

At the time of designation, aspiration pneumonitis requiring intubation and mechanical ventilation affected approximately 1.1 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of around 55,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

#### **What treatments are available?**

No satisfactory methods exist that were authorised at the time of application. The current treatment of the condition included immediate tracheal suction, which however is not able to treat the chemical burn on the trachea (or reach the bronchioli). Although there is a consensus that mechanical ventilation is mandatory in the condition, no treatment is of proven effectiveness in the condition.

#### **How is this medicine expected to work?**

Lusupultide is an artificial substitute for a naturally occurring liquid film, called surfactant, which is present on the lining of the airways and the alveoli in the lungs. Surfactant is necessary for the normal

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\*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 498,000,000 (Eurostat 2006).

function of the lungs. There is evidence that in aspiration pneumonitis the natural surfactant film is damaged because of the direct action of the acid gastric content, and also because of the secondary inflammatory process that occurs in the condition. Lusupultide is expected to help to restore the natural surfactant film, thus improving the oxygenation of the blood in these patients, with reduced duration of the condition.

#### **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, a clinical trial in patients with aspiration pneumonitis requiring intubation and mechanical ventilation was ongoing.

Lusupultide was not authorised anywhere worldwide for aspiration pneumonitis requiring intubation and mechanical ventilation.

Lusupultide is designated in the European Union as an orphan medicinal product for the treatment of acute respiratory distress syndrome (ARDS). In the United States orphan drug status was granted on 3 April 2000 for the treatment of Adult Respiratory Distress Syndrome, and 18 September 2006 for the treatment of pneumonia or aspiration of gastric contents leading to intubation, mechanical ventilation, and severe oxygenation impairment.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 31 May 2007 a positive opinion recommending the grant of the above-mentioned designation.

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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 Community) 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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**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	Lusupultide	Treatment of aspiration pneumonitis requiring intubation and mechanical ventilation
Bulgarian	Лусупултид	Лечение на аспирационен пневмонит, изискващ интубиране и механично вентилиране
Czech	Lusupultid	Léčba aspirační pneumonitidy vyžadující intubaci a mechanickou ventilaci
Danish	Lusupultid	Behandling af aspirationspneumoni som kræver intubation og mekanisk ventilation
Dutch	Lusupultide	Behandeling van aspiratiepneumonitis welke intubatie en mechanische beademing vereist
Estonian	Lusupultiid	Aspiratsioonipneumoniidi, mis vajab intubatsiooni ja mehhaanilist ventilatsiooni, ravi.
Finnish	Lusupultidi	Aspiraatiopneumoniitin, joka vaatii intubaation ja hengityskonehoidon, hoito
French	Lusupultide	Traitement de la pneumopathie d'inhalation nécessitant intubation et ventilation mécanique
German	Lusupultide	Behandlung der Aspirationspneumonitis, die zu Intubation und mechanischer Beatmung führt
Greek	Lusupultide	Θεραπεία της πνευμονίτιδας από αναρρόφηση που απαιτεί διασωλήνωση και μηχανικό αερισμό
Hungarian	Lusupultide	Intubációt és gépi lélegeztetést igénylő aspirációs pneumonitis kezelése
Italian	Lusupultide	Tattamento della polmonite da aspirazione, necessitante intubazione e ventilazione meccanica
Latvian	Lusupultīds	Aspirācijas pneimonīta, kuram nepieciešama intubācija un mehāniska ventilācija ārstēšana
Lithuanian	Lusupultidas	Aspiracinio pneumonito, reikalaujančio intubacijos ir mechaninės ventiliacijos, gydymas
Maltese	Lusupultide	Kura tal-pulmonite ikkawżata mill-ġbid man-nifs tal-kontenut gastriku li jehtieġilha intubazzjoni u ventilazzjoni mekkanika
Polish	Lusupultide	Leczenie zachlystowego zapalenia płuc z koniecznością intubacji i wentylacji mechanicznej
Portuguese	Lusupultida	Tratamento da pneumonite por aspiração que requer intubação e ventilação mecânica
Romanian	Lusupultid	Tratamentul pneumoniei de aspirație care necesită intubație și ventilație mecanică
Slovak	Lusupultid	Liečba aspiračnej pneumonitídy, ktorá vyžaduje intubáciu a mechanickú ventiláciu
Slovenian	Lusupultid	Zdravljenje aspiracijskega pnevmonitisa, ki zahteva intubacijo in umetno ventilacijo
Spanish	Lusupultida	Tratamiento de la aspiración del contenido gástrico que requiere intubación y ventilación mecánica
Swedish	Lusupultid	Behandling av aspirations-pneumonit som kräver intubation och mekanisk ventilation

Norwegian	Lusupultid	Behandling av aspirasjonspneumonittsom krever intubasjon og respiratorbehandling
Icelandic	Lúsúpúltíð	Meðferð við ásvelgingar lungnabólgu sem krefst barkaþræðingu og vélrænnar öndunarhjálp