



Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of aviptadil for the treatment of acute lung injury

On 28 August 2006, orphan designation (EU/3/06/395) was granted by the European Commission to mondoBIOTECH Laboratories, Lichtenstein, for aviptadil for the treatment of acute lung injury.

What is acute lung injury?

Acute lung injury (ALI) is a condition in which the capacity of the lungs to oxygenate the blood is greatly reduced, even if oxygen is administered in high concentrations. The most severe form of acute lung injury is known as adult respiratory distress syndrome.

The lungs are full of tiny sac-like structures called alveoli. It is in the alveoli where oxygen and carbon dioxide are exchanged between the air we inhale and the blood that perfuses the lungs in tiny vessels called capillaries. When the lungs are injured, for instance by an infection, blood and fluids begin to leak from the capillaries, into the alveoli. Subsequently, air cannot enter the alveoli and pass into the blood, which means that the normal functions of the lung tissue are impaired. This will lead to inflammation (a response to the injury caused to the tissue that includes swelling) and progressive formation of scar tissue in the walls of the alveoli. The affected patient will find it more and more difficult to breathe. There are many possible causes of ALI, such as inhaling high concentrations of smoke, toxins, or oxygen; severe burns; blood infections (the most common cause); lung infections; or trauma to other parts of the body. 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 less than 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of less than 92,000 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 medicinal products were authorised in the Community for the treatment of acute lung injury, at the time of submission of the application for orphan drug designation. The treatment options for acute lung injury were limited to life-support and artificial ventilation with high concentrations of oxygen. Antibiotics were also used to treat the infections and reduce the inflammation.

What is the stage of development of this medicine?

Aviptadil is a chemically produced substance that is identical to a natural hormone (a protein that circulates in the human blood), called vasoactive intestinal peptide. Aviptadil is a vasodilator (it is able to widen blood vessels) and it can lower blood pressure, if administered intravenously. However, its

*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 459,700,000 (Eurostat 2004).

mechanism of action in acute lung injury is related to its ability to influence the immune system (the body's natural defence system). This may decrease the inflammatory processes seen in acute lung injury. Aviptadil is also expected to be anti-apoptotic (it can prevent cells from dying). According to the sponsor, these functions of aviptadil will help prevent the lungs from being irreversibly damaged in acute lung injury.

What is the stage of development of this medicinal product?

The effects of aviptadil 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.

Aviptadil was not authorised anywhere worldwide for the treatment of acute lung injury, at the time of submission. Orphan designation of aviptadil was granted in the United States for respiratory distress syndrome and for pulmonary arterial hypertension.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 12 July 2006 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;
- 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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Patients' association contact point: Not available

**Translations of the active ingredient and indication in all official EU languages,
Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Aviptadil	Treatment of acute lung injury
Czech	Aviptadil	Léčba akutního poškození plic
Danish	Aviptadil	Behandling af akut lungeskade syndrom
Dutch	Aviptadil	Behandeling van acute longbeschadiging
Estonian	Aviptadil	Ägeda kopsuvigastuse ravi
Finnish	Aviptadiili	Akuutin keuhkovamman hoito
French	Aviptadil	Traitement de l'agression pulmonaire aiguë
German	Aviptadil	Behandlung des akuten Lungenversagens
Greek	Aviptadil	Θεραπεία της οξείας πνευμονικής βλάβης
Hungarian	Aviptadil	Akut tüdőkárosodás kezelése
Italian	Aviptadil	Trattamento della Lesione polmonare acuta
Latvian	Aviptadils	Akūta plaušu bojājuma ārstēšana
Lithuanian	Aviptadilis	Ūmaus plaučių pažeidimo gydymas
Polish	Awiptadil	Leczenie ostrego uszkodzenia płuc
Portuguese	Aviptadil	Tratamento da lesão pulmonar aguda
Slovak	Aviptadil	Liečba akútneho poškodenia pľúc
Slovenian	Aviptadil	Zdravljenje akutne poškodbe pljuč
Spanish	Aviptadil	Tratamiento de la lesión pulmonar aguda
Swedish	Aviptadil	Behandling av akut lungskada

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Norwegian	Aviptadil	Behandling av akutt lungeskade
Icelandic	Aviptadil	Til meðferðar á bráðum lungnaskaða