



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Adrenomedullin for the treatment of acute lung injury

On 9 June 2010, orphan designation (EU/3/10/744) was granted by the European Commission to Prof Dr Stefan Hippenstiel, Germany, for adrenomedullin for the treatment of acute lung injury.

The sponsorship was transferred to Mondobiotec Laboratories AG, Liechtenstein, in September 2011.

#### **What is acute lung injury?**

Acute lung injury is damage to the lungs that causes fluid to leak into the alveoli (the tiny air sacs in the lungs where the exchange of oxygen between the air and the blood takes place). This results in the blood not receiving enough oxygen. Patients with acute lung injury have difficulty breathing, tachycardia (rapid heart beat), and their lips, fingers and toes become slightly blue in colour. There are many possible causes of acute lung injury, such as inhaling high amounts of smoke or toxic gases, severe burns, near-drowning, drug overdose, blood or lung infections, inflammation of the pancreas, lung contusion (bruising) and trauma to another part of the body.

Acute lung injury is a life-threatening disease because it causes breathing problems, which can be fatal.

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

At the time of designation, acute lung injury affected less than 3.4 people in 10,000 per year in the European Union (EU)\*. This is equivalent to a total of fewer than 172,000 people per year, and was considered to be below the threshold for orphan designation. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

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

At the time of designation, the main treatment for acute lung injury was mechanical ventilation (using a mask or a tube inserted into the airways to help the patient to breathe). Patients were also given antibiotics to treat infections, medicines to control fluids in the body and painkillers. In some cases,

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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 506,500,000 (Eurostat 2010).



prednisolone (a steroid anti-inflammatory medicine) was used, but this was only authorised for lung problems in the United Kingdom.

The sponsor has provided sufficient information to show that adrenomedullin might be of significant benefit for patients with acute lung injury because it works in a different way to existing treatments and early studies show that it might improve the outcome of patients with this condition. These assumptions will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

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

Adrenomedullin is a natural peptide (a short chain of amino acids) that is found in the blood. It is thought to work by attaching to receptors in the small blood vessels in the lungs, which has a number of effects including causing the blood vessels to widen. In patients with acute lung injury, adrenomedullin is expected to lower the blood pressure in the lungs, reduce inflammation and reduce the leakage of fluid from the blood vessels into the lung tissue. This is expected to help the lungs to work normally and oxygenate the blood.

This medicine is made of adrenomedullin produced via chemical processes. It is expected to be given to patients by infusion (drip into a vein).

### **What is the stage of development of this medicine?**

The effects of adrenomedullin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with adrenomedullin in patients with acute lung injury had been started by the sponsor, but results of clinical studies with adrenomedullin were available from the published literature.

At the time of submission, adrenomedullin was not authorised anywhere in the EU for acute lung injury or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 March 2010 recommending the granting of this 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 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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Adrenomedullin	Treatment of acute lung injury
Bulgarian	Адреномедулин	Лечение на остра белодробна недостатъчност
Czech	Adrenomedulin	Léčba akutního poškození plic
Danish	Adrenomedullin	Behandling af akut lungeskade syndrom
Dutch	Adrenomedulline	Behandeling van acute longbeschadiging
Estonian	Adrenomedulliin	Ägeda kopsuvigastuse ravi
Finnish	Adrenomedulliini	Akuutin keuhkovamman hoito
French	Adrénomédulline	Traitement de l'agression pulmonaire aiguë
German	Adrenomedullin	Behandlung des akuten Lungenversagens
Greek	Αδρενομεντουλίνη	Θεραπεία της οξείας πνευμονικής βλάβης
Hungarian	Adrenomedullin	Akut tüdőkárosodás kezelése
Italian	Adrenomedullina	Trattamento della Lesione polmonare acuta
Latvian	Adrenomedulīns	Akūta plaušu bojājuma ārstēšana
Lithuanian	Adrenomedulinas	Ūmaus plaučių pažeidimo gydymas
Maltese	Adrenomedullin	Kura ta' korriment akut fil-pulmun
Polish	Adrenomedulina	Leczenie ostrego uszkodzenia płuc
Portuguese	Adrenomedulina	Tratamento da lesão pulmonar aguda
Romanian	Adrenomedulină	Tratamentul leziunilor pulmonare acute
Slovak	Adrenomedulín	Liečba akútneho poškodenia pľúc
Slovenian	Adrenomedulin	Zdravljenje akutne poškodbe pljuč
Spanish	Adrenomedulina	Tratamiento de la lesión pulmonar aguda
Swedish	Adrenomedullin	Behandling av akut lungskada
Norwegian	Adrenomedullin	Behandling av akutt lungeskade
Icelandic	Adrenómedúllín	Til meðferðar á bráðum lungnaskaða

<sup>1</sup> At the time of designation