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## **Committee for Orphan Medicinal Products**

### **Public summary of positive opinion for orphan designation of human tumour necrosis factor alpha-derived peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro- Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys for the treatment of acute lung injury**

On 8 October 2009, orphan designation (EU/3/09/677) was granted by the European Commission to APEPTICO Forschung und Entwicklung GmbH, Austria, for human tumour necrosis factor alpha-derived peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys for the treatment of acute lung injury.

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

Acute lung injury is damage to the lungs in which fluid leaks into the alveoli, the tiny air sacs in the lungs where the exchange of oxygen between air and 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 bluish 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) or trauma to other parts of the body.

Acute lung injury is a life-threatening disease because of problems with breathing.

#### **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, which was considered to be below the threshold for orphan designation. This is based on the information provided by the sponsor and 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, prednisolone (an anti-inflammatory medicine authorised in the UK) was used.

The sponsor has provided sufficient information to show that human tumour necrosis factor alpha-derived peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys might be of significant benefit for patients with acute lung injury because it works in a different way to existing treatments and early studies indicate that it might improve the treatment 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.

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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 504,800,000 (Eurostat 2009).

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

Human tumour necrosis factor alpha-derived peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys is a part of a human protein, called tumour necrosis factor alpha (TNF $\alpha$ ). It is produced in the laboratory. This medicine corresponds to the part of TNF $\alpha$  that is thought to be responsible for activating some channels in the lungs to stimulate fluid absorption. In patients with acute lung injury, this medicine given by inhalation is expected to stimulate fluid clearance from the alveoli, helping the lungs to function normally and oxygenate the blood. Unlike TNF $\alpha$ , this medicine is not expected to cause inflammation.

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

The effects of this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with acute lung injury had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for acute lung injury or designated as 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 8 July 2009 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 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:**

Sponsor's contact details:

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Patient associations' contact points:

None available.

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

<b>Language</b>	<b>Active ingredient</b>	<b>Indication</b>
English	Human tumour necrosis factor alfa-derived peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Treatment of acute lung injury
Bulgarian	Човешки тумор некротизиращ фактор алфа-придобит пептид Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Лечение на остра белодробна недостатъчност
Czech	lidský faktor nekrotizující nádory alfa derivát peptidu Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Léčba akutního poškození plic
Danish	Humant tumor nekrose faktor alfa afledt peptid Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Behandling af akut lungeskade syndrom
Dutch	Humaan tumornecrosefactor alfa-afgeleid peptide Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Behandeling van acute longbeschadiging
Estonian	Inimese tuumornekroosifaktor alfa-st tulenev peptiid Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Ägeda kopsuvigastuse ravi
Finnish	Ihmisen tuumorinekroositekijä alfa-st johdettu peptidi Kys-Gly-Gln-Arg-Glu-Tre-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Try-Tyr-Kys	Akuutin keuhkovamman hoito
French	Peptide dérivé du facteur humain de nécrose tumorale (TNF) alpha Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Traitement de l'agression pulmonaire aiguë
German	Humanes, von Tumornekrosefaktor-alpha abgeleitetes Peptid	Behandlung des akuten Lungenversagens
Greek	ανθρώπινος παράγοντας νέκρωσης όγκων α παράγωγο πεπτιδιο Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Θεραπεία της οξείας πνευμονικής βλάβης
Hungarian	humán tumornekrózis faktor alfa derivált peptid Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Akut tüdőkárosodás kezelése
Italian	Peptide derivato dal fattore umano di necrosi tumorale alfa Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Trattamento della lesione polmonare acuta
Latvian	Cilvēka tumora nekrozes faktora alfa atvasinātais peptīds Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Akūta plaušu bojājuma ārstēšana
Lithuanian	Žmogaus naviko nekrozės faktoriaus alfa išvestas peptidas Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Ūmaus plaučių pažeidimo gydymas
Maltese	Peptide mnissel mill-fattur uman tan-nekrosi tat-tumur alfa Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Kura ta' korriment akut fil-pulmun
Polish	Peptyd pochodny z ludzkiego czynnika martwicy nowotworu alfa Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Leczenie ostrego uszkodzenia płuc
Portuguese	Péptido Derivado do Factor de Necrose Tumoral	Tratamento da lesão pulmonar

	Humano alfa Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	aguda
Romanian	Peptidă derivată din Factorul uman de necroză tumorală alfa Cis-Gli-Gln-Arg-Glu-Tr-Pro-Glu-Gli-Ala-Glu-Ala-Lis-Pro-Trp-Tir-Cis	Tratamentul leziunilor pulmonare acute
Slovak	Peptid odvodený od ľudského faktora nekrotizujúceho nádory alfa Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Liečba akútneho poškodenia pľúc
Norwegian	Human tumornekrosefaktor alfa avledet peptid Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Behandling av akutt lungeskade
Icelandic	Manna æxlisdrepsþáttur alfa afleidd peptíð Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Til meðferðar á bráðum lungnaskaða