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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation Asp-Arg-Val-Tyr-Ile-His-Pro for the treatment of acute lung injury

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 10 October 2012, orphan designation (EU/3/12/1047) was granted by the European Commission to Tarix Pharmaceuticals Limited, Cyprus, for Asp-Arg-Val-Tyr-Ile-His-Pro for the treatment of acute lung injury.

The sponsorship was transferred to Gregory Fryer Associates Ltd, United Kingdom, in June 2013.

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 heartbeat), 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, and is associated with multiple organ failure and sepsis (when bacteria and their toxins circulate in the blood and damage the organs).



What is the estimated number of patients?

At the time of designation, acute lung injury affected not more than 3.4 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 173,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. 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 treatment for the causes of lung injury including antibiotics to treat infections, and medicines to control fluids in the body and symptoms such as pain. In the United Kingdom, prednisolone (a steroid anti-inflammatory medicine) is authorised for lung problems, and may be given in some cases of acute lung injury, but this is not a usual treatment.

The sponsor has provided sufficient information to show that Asp-Arg-Val-Tyr-Ile-His-Pro 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 be more effective at treating the condition. This assumption 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?

Asp-Arg-Val-Tyr-Ile-His-Pro is a peptide (a short chain of amino acids, the building blocks of proteins), which is a copy of a naturally occurring peptide called angiotensin (1-7) found in the blood. In patients with acute lung injury, Asp-Arg-Val-Tyr-Ile-His-Pro is expected to restore the balance of the 'renin-angiotensin system', a hormonal system that regulates blood pressure and fluid balance, which is believed to be over-activated in the lungs of these patients. This medicine is thought to work by attaching to receptors in the small blood vessels in the lungs, which has a number of effects that reduce the local actions of the renin-angiotensin system.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of Asp-Arg-Val-Tyr-Ile-His-Pro in experimental models was ongoing.

At the time of submission, no clinical trials with this medicine in patients with acute lung injury had been started.

At the time of submission, Asp-Arg-Val-Tyr-Ile-His-Pro 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 5 September 2012 recommending the granting of this designation.

*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. At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

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

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Asp-Arg-Val-Tyr-Ile-His-Pro	Treatment of acute lung injury
Bulgarian	Asp (аспаргинова киселина) – Arg (аргинин) – Val (валин) – Tyr (тирозин) – Ile (изолевцин) - His (хистидин), Pro (пролин)	Лечение на остра белодробна недостатъчност
Czech	Asp-Arg-Val-Tyr-Ile-His-Pro	Léčba akutního poškození plic
Danish	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling af akut lungeskade syndrom
Dutch	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandeling van acute longbeschadiging
Estonian	Asp-Arg-Val-Tyr-Ile-His-Pro	Ägeda kopsuvigastuse ravi
Finnish	Asp-Arg-Val-Tyr-Ile-His-Pro	Akuutin keuhkovamman hoito
French	Asp-Arg-Val-Tyr-Ile-His-Pro	Traitement de l'agression pulmonaire aiguë
German	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandlung des akuten Lungenversagens
Greek	Asp-Arg-Val-Tyr-Ile-His-Pro	Θεραπεία της οξείας πνευμονικής βλάβης
Hungarian	Asp-Arg-Val-Tyr-Ile-His-Pro	Akut tüdőkárosodás kezelése
Italian	Asp-Arg-Val-Tyr-Ile-His-Pro	Trattamento della Lesione polmonare acuta
Latvian	Asp-Arg-Val-Tyr-Ile-His-Pro	Akūta plaušu bojājuma ārstēšana
Lithuanian	Asp-Arg-Val-Tyr-Ile-His-Pro	Ūmaus plaučių pažeidimo gydymas
Maltese	Asp-Arg-Val-Tyr-Ile-His-Pro	Kura ta' korriment akut fil-pulmun
Polish	Asp-Arg-Val-Tyr-Ile-His-Pro	Leczenie ostrego uszkodzenia płuc
Portuguese	Asp-Arg-Val-Tyr-Ile-His-Pro	Tratamento da lesão pulmonar aguda
Romanian	Asp-Arg-Val-Tyr-Ile-His-Pro	Tratamentul leziunilor pulmonare acute
Slovak	Asp-Arg-Val-Tyr-Ile-His-Pro	Liečba akútneho poškodenia pľúc
Slovenian	Asp-Arg-Val-Tyr-Ile-His-Pro	Zdravljenje akutne poškodbe pljuč
Spanish	Asp-Arg-Val-Tyr-Ile-His-Pro	Tratamiento de la lesión pulmonar aguda
Swedish	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling av akut lungskada
Norwegian	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling av akutt lungeskade
Icelandic	Asp-arg-val-týr-íle-his-pró	Til meðferðar á bráðum lungnaskaða

¹ At the time of designation