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EMA/COMP/167823/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ubenimex for the treatment of pulmonary arterial hypertension

On 21 March 2016, orphan designation (EU/3/16/1638) was granted by the European Commission to Eiger Biopharmaceuticals Europe Limited, United Kingdom, for ubenimex for the treatment of pulmonary arterial hypertension.

What is pulmonary arterial hypertension?

Pulmonary arterial hypertension is a condition in which patients have abnormally high blood pressure in the arteries that supply the lungs. In pulmonary arterial hypertension, the muscles in the walls of the arteries in the lungs become thicker and the arteries become narrower, making it harder for blood to flow to the lungs.

Pulmonary arterial hypertension is a long-term debilitating and life-threatening condition that shortens patients' life expectancy because it may lead to difficulty breathing and heart failure.

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

At the time of designation, pulmonary arterial hypertension affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 103,000 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?

Several medicines were authorised for the treatment of pulmonary arterial hypertension in the EU at the time of designation. They included ambrisentan, bosentan, epoprostenol, iloprost, macitentan, riociguat, sildenafil, tadalafil and treprostinil. Surgery was also used in some patients to carry out a lung transplant or atrial septostomy (where a small hole is created between the upper two chambers of the heart, the atria).

^{*}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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

The sponsor has provided sufficient information to show that ubenimex might be of significant benefit for patients with pulmonary arterial hypertension. Experimental studies show that it may improve survival and the ability of the heart to send blood to the lungs more than another medicine, sildenafil. 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?

Many of the symptoms of pulmonary arterial hypertension are thought to be caused by part of the body's defence response working incorrectly, leading to inflammation. Ubenimex blocks the activity of a protein called LTA₄ hydrolase. This protein normally promotes the production of LTB₄, which is involved in inflammation and is found at high levels in the lungs of patients with pulmonary arterial hypertension. Blocking this protein is therefore expected to reduce inflammation and thus improve symptoms of pulmonary arterial hypertension.

What is the stage of development of this medicine?

The effects of ubenimex have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with ubenimex in patients with pulmonary arterial hypertension had been started.

At the time of submission, ubenimex was authorised in Japan, Korea and China for cancer.

At the time of submission, ubenimex was not authorised anywhere in the EU for pulmonary arterial hypertension. Orphan designation of ubenimex had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 February 2016 recommending the granting of this 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 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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	Ubenimex	Treatment of pulmonary arterial hypertension
Bulgarian	Убенимекс	Лечение на белодробна артериална хипертония
Croatian	Ubenimeks	Liječenje plućne arterijske hipertenzije
Czech	Ubenimex	Léčba plicní arteriální hypertenze
Danish	Ubenimex	Behandling af pulmonal arteriel hypertension
Dutch	Ubenimex	Behandeling van pulmonale arteriële hypertensive
Estonian	Ubenimeks	Arteriaalse pulmonaalhüpertensiooni ravi
Finnish	Ubenimeksi	Keuhkoverenkierron hypertension hoito
French	Ubenimex	Traitement de l'hypertension artérielle pulmonaire
German	Ubenimex	Behandlung der pulmonalen arteriellen Hypertonie
Greek	Ουμπενιμέξη	Θεραπεία της πνευμονικής αρτηριακής υπέρτασης
Hungarian	Ubenimex	Pulmonáris arteriális hipertónia kezelése
Italian	Ubenimex	Trattamento dell'ipertensione arteriosa polmonare
Latvian	Ubenimeks	Plaušu arteriālās hipertensijas ārstēšana
Lithuanian	Ubenimeksas	Plaučių arterinės hipertenzijos gydymas
Maltese	Ubenimex	Kura ta' pressjoni arterjali pulmonari għolja
Polish	Ubenimex	Leczenie tętniczego nadciśnienia płucnego
Portuguese	Ubenimex	Tratamento da hipertensão arterial pulmonar
Romanian	Ubenimex	Tratamentul hipertensiunii arteriale polmonare
Slovak	Ubenimex	Liečba pľúcnej arteriálnej hypertenzie vrátane
Slovenian	Ubenimeks	Zdravljenje pljučne arterijske hipertenzije vključno
Spanish	Ubenimex	Tratamiento de la hipertensión arterial pulmonar
Swedish	Ubenimex	Behandling av pulmonell arteriell hypertension
Norwegian	Ubenimex	Behandling av pulmonal arteriell hypertensjon
Icelandic	Úbenimex	Meðferð við háþrýstingi í lungnablóðrás

¹ At the time of designation