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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Beraprost sodium (modified release tablet) for the treatment of pulmonary arterial hypertension

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Rev.1: transfer of sponsorship	21 March 2012
Rev.2: administrative update	30 April 2012
Rev.3: sponsor's name and address change	5 April 2013
Rev.4: sponsor's change of address	10 March 2015
Rev.5: sponsor's change of address	22 May 2015
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 July 2008, orphan designation (EU/3/08/554) was granted by the European Commission to Lung Rx Limited, United Kingdom, for beraprost sodium (modified release tablet) for the treatment of pulmonary arterial hypertension.

The sponsorship was transferred to IDEA Innovative Drugs European Associates Limited, United Kingdom, in February 2012.

In December 2012, Innovative Drug European Associates Limited changed name to IDEA Innovative Drug European Associates Limited.

What is pulmonary arterial hypertension?

Pulmonary arterial hypertension is a disorder of the blood vessels leading to surrounding the lungs, in which the blood pressure in the pulmonary artery (the large blood vessel that carries blood from the heart to the lungs) becomes high. It is caused by the walls of the lung's blood vessels become thicker, harder and less elastic, making it more difficult for the blood to move through the vessels. The cause of these changes in the vessels is unknown. In the long term, the high blood pressure causes damage to the heart and symptoms such as shortness of breath, tiredness, chest pain, weakness, coughing,

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wheezing and swelling. Pulmonary arterial hypertension is a debilitating disease that is long-lasting and may be life-threatening.

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 around 101,000 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 submission of the application for orphan drug designation, several medicines were authorised in the European Union (EU) for the treatment of pulmonary arterial hypertension.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that beraprost sodium (modified release tablet) might be of potential significant benefit for the treatment of pulmonary arterial hypertension. This is mainly because of the expected clinical effect of the medicine in relation to and its mode of administration, which could make the treatment more convenient for patients. 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?

Beraprost sodium is a molecule that is very similar to prostacyclin, a natural substance that causes the blood vessels to dilate (widen). In patients with pulmonary arterial hypertension, beraprost sodium is expected to act in the same way as prostacyclin to dilate the pulmonary arteries, lowering the blood pressure in the pulmonary artery and improving the symptoms of the disease.

Beraprost sodium is to be available as modified release tablets. This means that the tablets have been made to release beraprost sodium over a few hours. This would allow the patient to be treated using an oral treatment that can be taken twice a day.

What is the stage of development of this medicine?

The effects of beraprost sodium (modified release tablet) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, the evaluation of the effects of beraprost sodium (modified release tablet) in experimental models was ongoing.

Beraprost is authorised in different countries for indications including pulmonary arterial hypertension. A modified release form of beraprost has been available in Japan for pulmonary arterial hypertension since 2007.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 14 May 2008 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 502,800,000 (Eurostat 2008).

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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Beraprost sodium (modified release tablet)	Treatment of pulmonary arterial hypertension
Bulgarian	Натриев берапрост (Таблетки с изменено освобождаване)	Лечение на белодробна артериална хипертония
Czech	Beraprost sodný (upravené uvolňovací tablety)	Léčba plicní arteriální hypertenze
Danish	Natriumberaprost (tablet med modificeret udløsning)	Behandling af pulmonal arteriel hypertension
Dutch	Beraprost natrium (tablet met gereguleerde afgifte)	Behandeling van pulmonale arteriële hypertensie
Estonian	Beraprostnaatrium (modifitseeritud vabanemisega tablett)	Arteriaalse pulmonaalhüpertensiooni ravi
Finnish	Beraprostinatrium (depottabletti)	Keuhkoverenkierron hypertension hoito
French	Béraprost sodium (comprimé à libération modifiée)	Traitement de l'hypertension artérielle pulmonaire
German	Beraprost-Natrium (Tablette mit veraenderter Wirkstofffreisetzung)	Behandlung der pulmonalen arteriellen Hypertonie
Greek	Νατριούχος βεραπρόστη (δισκίο τροποποιημένης απελευθέρωσης)	Θεραπεία της πνευμονικής αρτηριακής υπέρτασης
Hungarian	Beraprost-nátrium (módosított hatóanyagleadású tabletta)	Pulmonáris arteriális hipertónia kezelése
Italian	Beraprost sodio (compressa a rilascio modificato)	Trattamento dell'ipertensione arteriosa polmonare
Latvian	Nātrija beraprosts (mainīgas darbības tabletes)	Plaušu arteriālās hipertensijas ārstēšana
Lithuanian	Beraprosto natrio druska (modifikuota atpalaiduota tabletė)	Plaučių arterinės hipertenzijos gydymas
Maltese	Beraprost sodium (pillola li terħi l-mediċina b'mod modifikat)	Kura ta' pressjoni arterjali pulmonari għolja
Polish	Beraprost sodu (tabletki o zmodyfikowanym uwalnianiu)	Leczenie tętniczego nadciśnienia płucnego
Portuguese	Beraprost sódico (comprimido de libertação modificada)	Tratamento da hipertensão arterial pulmonar
Romanian	Beraprost sodic (comprimate cu eliberare modificată)	Tratamentul hipertensiunii arteriale polmonare
Language	Active ingredient	Indication
Slovak	Beraprost sodná soľ (tableta s riadeným uvoľňovaním)	Liečba pľúcnej arteriálnej hypertenzie vrátane
Slovenian	Natrijev beraprost (tablete s prirejenim sproščanjem)	Zdravljenje pljučne arterijske hipertenzije vključno
Spanish	Beraprost sódico (comprimido de liberación modificada)	Tratamiento de la hipertensión arterial pulmonar

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Beraprostnatrium (tablett med modifierad frisättning)	Behandling av pulmonell arteriell hypertension
Norwegian	Beraprostnatrium (tablett med modifisert frisetting)	Behandling av pulmonal arteriell hypertensjon
Icelandic	Beraprost natríum (tafla með aðlagaðri losun)	Meðferð við háþrýstingi í lungnablóðrás