



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2004 on request of the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF

beraprost sodium for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension

On 18 September 2001, orphan designation (EU/3/01/053) was granted by the European Commission to Aventis Pharma SA, France, for beraprost sodium for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension.

What is pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension?

Pulmonary arterial hypertension is a rare blood vessel disorder of the lung in which the pressure in the pulmonary artery (the blood vessel that leads from the heart to the lungs) rises above normal levels. An increase of the number of smooth muscle cells in the walls of small lung arteries (a phenomenon called proliferation) that are remodelling the vessels, may lead to obstructions in the microcirculation, which will then lead to an increase in the blood pressure.

Chronic thromboembolic pulmonary hypertension is a complication representing less than 1% of all cases of acute pulmonary embolism (the sudden blocking of a lung artery by a clot or foreign material which has been brought to its site by the blood current), which directly leads to pulmonary hypertension. Pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension are chronically debilitating and life-threatening.

What are the methods of treatment available?

One medicinal product was authorised for the treatment of pulmonary arterial hypertension in the Community at the time of submission of the application for orphan drug designation.

Beraprost sodium might be of potential significant benefit for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension mainly because it might contribute to patient care because of the new route of administration. This potential benefit will have to be confirmed at the time of marketing authorisation and this will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension was considered to affect about 26,000 in the European Union.

How is this medicinal product expected to act?

Beraprost sodium is a protein similar to a natural substance, prostacyclin, normally released by certain white blood cells (cells of the body's immune system). It is formulated in an oral tablet and might enlarge the diameter of the vessels, also on the pulmonary arteries. This effect is called vasodilatation.

Beraprost sodium might also help to diminish the blood clotting in the vessels, which might also help in the treatment of the condition.

What is the stage of development of this medicinal product?

The effects of beraprost sodium were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension were ongoing.

Beraprost sodium was not marketed anywhere worldwide for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension, at the time of submission. Orphan designation of beraprost sodium was granted in Japan and in the United states for the same condition.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 18 July 2001 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

Sponsor's contact details:

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Please note that beraprost sodium is now owned by Toray Industries, Japan.

*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Patients' associations contact points:

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Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Beraprost sodium	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Danish	Natriumberaprost	Behandling af pulmonal arteriel hypertension og kronisk tromboembolisk pulmonal hypertension
Dutch	Beraprost natrium	Behandeling van pulmonaire arteriële hypertensie en chronische thrombo-embolische pulmonaire hypertensie
Finnish	Beraprostinatrium	Pulmonaarihypertension ja kroonisesta tromboemboliasta johtuvan pulmonaarisen hypertension hoito
French	Béraprost sodium	Traitement de l'hypertension artérielle pulmonaire et de l'hypertension pulmonaire secondaire à une maladie thrombo-embolique chronique
German	Beraprost-Natrium	Behandlung der pulmonalen arteriellen Hypertonie und der chronischen thromboembolischen pulmonalen Hypertonie
Greek	Νατριούχος βεραπρόστη	Αγωγή της πνευμονικής αρτηριακής υπέρτασης και της χρόνιας θρομβοεμβολικής πνευμονικής υπέρτασης
Italian	Beraprost sodio	Trattamento dell'ipertensione arteriosa polmonare e dell'ipertensione tromboembolica polmonare cronica
Portuguese	Beraprost sódico	Tratamento da hipertensão pulmonar arterial e da hipertensão pulmonar tromboembólica crónica
Spanish	Beraprost sódico	Tratamiento de la hipertensión pulmonar y de la hipertensión pulmonar tromboembólica crónica
Swedish	Beraprostnatrium	Behandling av pulmonär arteriell hypertension och kronisk tromboembolisk pulmonalishypertension