



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Treprostinil sodium for the treatment of chronic thromboembolic pulmonary hypertension

On 8 February 2013, orphan designation (EU/3/13/1103) was granted by the European Commission to SciPharm S.a.r.L, Luxembourg, for treprostinil sodium for the treatment of chronic thromboembolic pulmonary hypertension.

What is chronic thromboembolic pulmonary hypertension?

Chronic thromboembolic pulmonary hypertension is a condition where there is high blood pressure in the arteries that supply the lungs. The increased blood pressure in the arteries of the lungs is caused by a blockage due to blood clots, which results in progressive narrowing of such arteries and a subsequent thickening in the muscles in the walls of the arteries, making it harder for the heart to pump blood to the lungs.

Chronic thromboembolic pulmonary hypertension is long-term debilitating due to symptoms such as fatigue and dyspnoea (difficulty breathing), which limit daily activities. If untreated it may also be life threatening due to the development of heart failure.

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

At the time of designation, chronic thromboembolic pulmonary hypertension affected between 0.10 and 0.52 in 10,000 people in the European Union (EU). This was equivalent to a total of between about 5,000 and 26,000 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).

^{*}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 509,000,000 (Eurostat 2013).



What treatments are available?

At the time of designation, no medicines were authorised in the EU specifically for chronic thromboembolic pulmonary hypertension. Some patients were treated with surgery or lung transplantation.

How is this medicine expected to work?

Treprostinil sodium is a molecule that is very similar to prostacyclin, a natural substance that dilates (widens) the blood vessels and prevents blood clotting. In patients with chronic thromboembolic pulmonary hypertension, treprostinil sodium is expected to act in the same way as prostacyclin to dilate the pulmonary arteries and prevent blood clotting, lowering the blood pressure in the pulmonary artery and improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of treprostinil sodium have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with treprostinil sodium initiated by the sponsor in patients with chronic thromboembolic pulmonary hypertension had been started.

Treprostinil sodium was authorised in the United States of America and in many EU Member States for the treatment of pulmonary arterial hypertension, which is another type of pulmonary hypertension.

At the time of submission, treprostinil sodium was not authorised anywhere in the EU for chronic thromboembolic pulmonary hypertension 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 9 January 2013 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:

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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	Treprostinil sodium	Treatment of chronic thromboembolic pulmonary hypertension
Bulgarian	Натриев трепростинил	Лечение на хронична тромбоемболична белодробна хипертония
Czech	Treprostinil sodný	Léčba chronické tromboembolické plicní hypertenze
Danish	Treprostinil natrium	Behandling af kronisk tromboembolisk pulmonal hypertension
Dutch	Treprostinil natrium	Behandeling van chronische trombo-embolische pulmonale hypertensie
Estonian	Treprostiinil-naatrium	Kroonilise trombemboolilise pulmonaalhüpertensiooni ravi
Finnish	Natriumtreprostiiniili	Kroonisen tromboembolisen keuhkohypertension hoito
French	Sodium de tréprostinil	Traitement de l'hypertension artérielle pulmonaire chronique thromboembolique
German	Treprostinil Natrium	Behandlung der chronischen thromboembolischen pulmonalen Hypertonie
Greek	Νατριούχος τρεπροστινίλη	Η θεραπεία της χρόνιας θρομβοεμβολικής πνευμονικής υπέρτασης
Hungarian	Treprostinil nátrium	Krónikus thromboemboliás pulmonalis hypertonia kezelése
Italian	Treprostinil sodio	Trattamento dell'ipertensione polmonare tromboembolica cronica
Latvian	Nātrija treprostinils	Ārstēšanai sabojājies hronisku trombembolisku plaušu hipertensija
Lithuanian	Natrio treprostinilis	Lėtinės tromboembolinės plaučių hipertenzijos gydymas
Maltese	Treprostinil sodium	Kura tal-pressjoni pulmonari trombo-embolika għolja
Polish	Treprostinil sodu	Leczenie przewlekłego zakrzepowo-zatorowego nadciśnienia płucnego
Portuguese	Treprostinil de sódio	Tratamento da hipertensão pulmonar tromboembólica crónica

¹ At the time of designation

Language	Active ingredient	Indication
Romanian	Treprostinil de sodiu	Tratamentul hipertensiunii pulmonare tromboembolice cronice
Slovak	Treprostinil sodný	Liečba chronickej tromboembolickej pľúcnej hypertenzie
Slovenian	Treprostinil natrijev	Zdravljenje kronične trombembolične neuporabno pljučna hipertenzija
Spanish	Treprostinil sódico	Tratamiento de la hipertensión pulmonar tromboembólica crónica
Swedish	Treprostinil natrium	Behandling av kronisk tromboembolisk pulmonell hypertension
Norwegian	Treprostinil natrium	Behandling av kronisk tromboembolisk pulmonal hypertensjon
Icelandic	Treprostíníl natríúm	Meðferð óskurðtæks langvinnum lungnaháþrýstingi vegna segareks