



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium nitrite for the treatment of pulmonary arterial hypertension

On 5 March 2012, orphan designation (EU/3/12/967) was granted by the European Commission to FGK Representative Service GmbH, Germany, for sodium nitrite for the treatment of pulmonary arterial hypertension.

What is pulmonary arterial hypertension?

Pulmonary arterial hypertension is a condition where there is high blood pressure in the arteries that supply the lungs. In patients with pulmonary arterial hypertension, there is a thickening of the muscles in the walls of the arteries and a narrowing of the arteries in the lungs, 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 heart failure and difficulty breathing.

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

At the time of designation, pulmonary arterial hypertension affected between 1.4 and 2.7 in 10,000 people in the European Union (EU)*. This is equivalent to a total of between 71,000 and 137,000 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, 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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).



The sponsor has provided sufficient information to show that sodium nitrite might be of significant benefit for patients with pulmonary arterial hypertension because early studies show that it works in a different way to existing treatments, which may improve the treatment of patients with pulmonary arterial hypertension. 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?

The medicine is expected to be available as a solution for inhalation by nebuliser (a small machine that changes a solution into an aerosol that the patient can breathe in). After inhalation, sodium nitrite is converted in the lungs into nitric oxide, which is expected to cause the vessels in the lungs to dilate (widen). This allows blood to flow more easily in the lungs to supply oxygen to the rest of the body and remove carbon dioxide, reducing pulmonary hypertension.

What is the stage of development of this medicine?

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

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 January 2012 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	Sodium nitrite	Treatment of pulmonary arterial hypertension
Bulgarian	Натриев нитрит	Лечение на белодробна артериална хипертония
Czech	Dusitan sodný	Léčba plicní arteriální hypertenze
Danish	Natriumnitrit	Behandling af pulmonal arteriel hypertension
Dutch	Natriumnitriet	Behandeling van pulmonale arteriële hypertensie
Estonian	Naatriumnitrit	Arteriaalse pulmonaalhüpertensiooni ravi
Finnish	Natriumnitriitti	Keuhkoverenkierron hypertension hoito
French	Nitrite de sodium	Traitement de l'hypertension artérielle pulmonaire
German	Natriumnitrit	Behandlung der pulmonalen arteriellen Hypertonie
Greek	Νιτρικό νάτριο	Θεραπεία της πνευμονικής αρτηριακής υπέρτασης
Hungarian	Nátrium-nitrit	Pulmonáris arteriális hipertónia kezelése
Italian	Sodio nitrito	Trattamento dell'ipertensione arteriosa polmonare
Latvian	Nātrija nitrīts	Plaušu arteriālās hipertensijas ārstēšana
Lithuanian	Natrio nitritas	Plaučių arterinės hipertenzijos gydymas
Maltese	Sodium Nitrite	Kura ta' pressjoni arterjali pulmonari għolja
Polish	Azotyn sodowy	Leczenie tętniczego nadciśnienia płucnego
Portuguese	Nitrito de sódio	Лечение на белодробна артериална хипертония
Romanian	Nitrit de sodiu	Léčba plicní arteriální hypertenze
Slovak	Dusitan sodný	Behandling af pulmonal arteriel hypertension
Slovenian	Natrijev nitrit	Behandeling van pulmonale arteriële hypertensie
Spanish	Nitrito de sodio	Arteriaalse pulmonaalhüpertensiooni ravi
Swedish	Natriumnitrit	Behandling av pulmonell arteriell hypertension
Norwegian	Natriumnitritt	Behandling av pulmonal arteriell hypertensjon
Icelandic	Natríumnítrít	Meðferð við háþrýstingi í lungnablóðrás

¹ At the time of designation