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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Bosentan for the treatment of idiopathic pulmonary fibrosis

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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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2014 on request of the Sponsor.

On 5 September 2008, orphan designation (EU/3/08/559) was granted by the European Commission to Actelion Registration Limited, United Kingdom, for bosentan for the treatment of idiopathic pulmonary fibrosis.

What is idiopathic pulmonary fibrosis?

Fibrosis is the formation of scar tissue that is part of the natural repair process of the body following tissue damage. Idiopathic pulmonary fibrosis consists of a chronic inflammation and progressive formation of fibrous tissue in the lungs. 'Idiopathic' means that the cause of the disease is unknown. The progressive formation of scars makes the lungs unable to work normally, reducing the transfer of oxygen from the air into the blood. Patients with idiopathic pulmonary fibrosis have a persistent cough, severe shortness of breath that gets worse and frequent lung infections.

Idiopathic pulmonary fibrosis is a long-term, debilitating and life-threatening disease.

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

At the time of designation, idiopathic pulmonary fibrosis affected approximately 1.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 95,000 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?

No satisfactory methods were authorised for the treatment of idiopathic pulmonary fibrosis at the time of application. Only medicines to relieve the symptoms of the disease were used, including corticosteroids (anti-inflammatory medicines) and medicines that reduce the activity of the immune system (the body's natural defences). In some patients, the disease may need to be treated with a lung transplant.

How is this medicine expected to work?

Bosentan is an 'endothelin receptor antagonist'. This means that it blocks the receptor to which a substance called 'endothelin-1' normally attaches itself. Endothelin-1 is a naturally occurring substance that is released from the endothelium (the cells lining the blood vessels). It has a wide range of effects, including causing fibrosis, cell proliferation and inflammation. By blocking the endothelin receptor, bosentan blocks the activity of endothelin-1, reducing the symptoms of idiopathic pulmonary fibrosis.

What is the stage of development of this medicine?

The effects of bosentan were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with idiopathic pulmonary fibrosis were ongoing.

At the time of submission, bosentan was not authorised anywhere in the world for the treatment of idiopathic pulmonary fibrosis or designated as an orphan medicinal product elsewhere for this condition.

The product was authorised in the Community for the treatment of pulmonary arterial hypertension and the treatment of digital ulcers in patients with systemic sclerosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 June 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).

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	Bosentan	Treatment of idiopathic pulmonary fibrosis
Bulgarian	Бозентан	Лечение на идиопатична белодробна фиброза
Czech	Bosentan	Léčba idiopatické plicní fibrózy
Danish	Bosentan	Behandling af idiopatisk lungefibrose
Dutch	Bosentan	Behandeling van idiopathische longfibrose
Estonian	Bosentaan	Idiopaatilise kopsufibroosi ravi
Finnish	Bosentaani	Idiopaattisen keuhkofibroosin hoito
French	Bosentan	Traitement de la fibrose pulmonaire idiopathique
German	Bosentan	Behandlung von Idiopathischer Pulmonaler Fibrose
Greek	Bosentan	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Bosentan	Idiopathiás tüdőfibrózis kezelése
Italian	Bosentan	Trattamento della fibrosi polmonare idiopatica
Latvian	Bosentāns	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	Bosentan	Idiopatinės plaučių fibrozės gydymas
Maltese	Bosentan	Kura tal-fibrożi pulmonari idjopatika
Polish	Bozentan	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Bosentan	Tratamento da fibrose pulmonar idiopática
Romanian	Bosentan	Tratamentul fibrozei pulmonare idiopatice
Slovak	Bosentan	Liečba idiopatickej pľúcnej fibrózy
Slovenian	Bosentan	Zdravljenje idiopatske pljučne fibroze
Spanish	Bosentán	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Bosentan	Behandling av idiopatisk lungfibros
Norwegian	Bosentan	Behandling av idiopatisk lungefibrose
Icelandic	Bósentan	Meðferð sjálfvakinnar bandvefsmyndunar í lungum

[1] At the time of designation