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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Macitentan 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.	

On 28 January 2010, orphan designation (EU/3/09/707) was granted by the European Commission to Actelion Registration Limited, United Kingdom, for macitentan for the treatment of idiopathic pulmonary fibrosis.

What is idiopathic pulmonary fibrosis?

Idiopathic pulmonary fibrosis is a long-term disease of the lungs characterised by the progressive formation of hard tissue in the lining (endothelium) of the lungs. 'Idiopathic' means that the cause of the disease is unknown. As the tissue becomes thicker and forms scars, the lungs become unable to work normally, reducing the transfer of oxygen from the air into the blood. Patients with idiopathic pulmonary fibrosis have a persistent cough, frequent lung infections and severe shortness of breath that worsens over time.

Idiopathic pulmonary fibrosis is a long-term debilitating disease that may be life-threatening because of problems with breathing.

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

At the time of designation, idiopathic pulmonary fibrosis affected approximately 2.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 137,000 people*, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and 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.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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What treatments are available?

At the time of application, there were no authorised medicines for the treatment of idiopathic pulmonary fibrosis. Only medicines to relieve the symptoms of the disease were available, 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?

Macitentan is expected to work as an 'endothelin receptor antagonist'. This means that it is expected to block 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. It has a wide range of effects, including causing fibrosis (scar tissue), cell proliferation and inflammation. By blocking the endothelin receptor, macitentan is expected to block the activity of endothelin-1, reducing the symptoms of idiopathic pulmonary fibrosis.

What is the stage of development of this medicine?

The effects of macitentan have been evaluated in experimental models.

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

At the time of submission, macitentan was not authorised anywhere in the EU for idiopathic pulmonary fibrosis. Orphan designation of macitentan had been granted in the United States of America for idiopathic pulmonary fibrosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 November 2009 recommending the granting of this designation.

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

[1] At the time of designation