1 September 2011
EMA/COMP/450736/2010 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ambrisentan for the treatment idiopathic pulmonary fibrosis

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in June 2011 on request of the sponsor.

On 1 October 2010, orphan designation (EU/3/10/790) was granted by the European Commission to Gilead Sciences International Ltd, United Kingdom, for ambrisentan for the treatment idiopathic pulmonary fibrosis.

What is idiopathic pulmonary fibrosis?

Idiopathic pulmonary fibrosis is a 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 the lungs gradually lose their ability to work properly.

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

At the time of designation, idiopathic pulmonary fibrosis affected less than 1.8 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 91,000 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 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 506,500,000 (Eurostat 2010).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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?

Ambrisentan is expected to work by blocking the endothelin type A 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 (increase in cell number) and inflammation. By blocking the endothelin type A receptor, ambrisentan is expected to block the activity of endothelin-1, thus reducing the symptoms of idiopathic pulmonary fibrosis.

What is the stage of development of this medicine?

The effects of ambrisentan have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with ambrisentan in patients with idiopathic pulmonary fibrosis was ongoing.

At the time of submission, ambrisentan was authorised in the EU for the treatment of pulmonary arterial hypertension.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2010 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:

Gilead Sciences International Ltd
Flowers Building
Granta Park
Abington
Cambridge CB21 6GT
United Kingdom
Telephone: + 44 1223 897 300
Telefax: + 44 1223 897 284
E-mail: ukmedinfo@gilead.com

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

[1] At the time of designation