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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pirfenidone for the treatment of idiopathic pulmonary fibrosis

First publication	1 July 2005
Rev.1: transfer of sponsorship	2 September 2009
Rev.2: information about Marketing Authorisation	23 March 2011
Rev.3: sponsor's name and address change	24 May 2011
Rev.4: sponsor's change of address	17 April 2012
Rev.5: sponsor's change of address	13 September 2013
Rev.6: transfer of sponsorship	12 March 2015
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 16 November 2004, orphan designation (EU/3/04/241) was granted by the European Commission to Uppsala Medical Information System AB, Sweden, for pirfenidone for the treatment of idiopathic pulmonary fibrosis.

The sponsorship was transferred to Intermune Europe Limited, United Kingdom, in May 2009. Intermune Europe Limited has changed its name to InterMune UK Limited in March 2011.

The sponsorship was transferred to Roche Registration Limited, United Kingdom, in February 2015.

What is idiopathic pulmonary fibrosis?

Fibrosis is the formation of scar tissue as part of the natural repair process of the body following tissue damage. Idiopathic pulmonary fibrosis consists of a chronic inflammation (a response of the body to the injury caused to the tissue) and progressive formation of fibrous tissue in the walls of the small chambers containing air in the lungs. Since the injury causing these changes is unknown, it is called idiopathic. The progressive formation of scars impairs the normal functions of lung tissue, which are to enable exchange of oxygen and carbon dioxide between air and blood. The symptoms developed are persistent cough and progressive severe shortness of breath.

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Idiopathic pulmonary fibrosis is a chronically debilitating and life threatening disease due to the progression of symptoms, severe respiratory complications and short life expectancy.

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

At the time of designation, idiopathic pulmonary fibrosis affected not more than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 139,000 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).

What treatments are available?

No satisfactory methods exist that were authorised at the time of application. Only symptomatic treatments to reduce the inflammation were used (corticosteroids and medicinal products that suppress the immune system) or for some patients lung transplantation was performed.

How is this medicine expected to work?

Scar formation (fibrosis) is regulated by several substances produced in the body during the inflammation that precedes fibrosis. These substances stimulate the so-called fibroblasts (the main cells responsible for formation of the fibrosis). Although it is not yet fully understood how pirfenidone acts in idiopathic pulmonary fibrosis, it could reduce the action on these substances and thereby reduce the scar formation.

What is the stage of development of this medicine?

The effects of pirfenidone were evaluated in experimental models.

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

Pirfenidone was not marketed anywhere worldwide for idiopathic pulmonary fibrosis, at the time of submission. Orphan designation of pirfenidone was granted in the United States for idiopathic pulmonary fibrosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2004 recommending the granting of this designation.

Update: pirfenidone (Esbriet) has been authorised in the EU since 28 February 2011. Esbriet is indicated in adults for the treatment of mild to moderate Idiopathic Pulmonary Fibrosis (IPF).

More information on Esbriet can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 464,200,000 (Eurostat 2004).

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 languages[1], Norwegian and Icelandic

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

[1] At the time of transfer of sponsorship