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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human pentraxin-2 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 17 July 2012, orphan designation (EU/3/12/1020) was granted by the European Commission to Appletree Europe S.a.r.l., Luxemburg, for recombinant human pentraxin-2 for the treatment of idiopathic pulmonary fibrosis.

The sponsorship was transferred to FGK Representative Service GmbH, Germany, in April 2014.

What is idiopathic pulmonary fibrosis?

Idiopathic pulmonary fibrosis is a long-term disease of the lungs characterised by the progressive deposition of collagen and fibrous tissue in the lungs. This causes the lung tissue to become inflamed, thick and form scars. As a result 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 shortness of breath that worsens over time.

Idiopathic pulmonary fibrosis is a life-threatening and long-term debilitating disease 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 not more than 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 76,000 people*, and is below

*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 509,000,000 (Eurostat 2012).

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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?

At the time of designation, Esbriet (pirfenidone) was the only medicine authorised in the EU to treat idiopathic pulmonary fibrosis.

The sponsor has provided sufficient information to show that recombinant human pentraxin-2 might be of significant benefit for patients with idiopathic pulmonary fibrosis because early studies in experimental models show that it works in a different way to existing treatments and might improve the outcome of patients with this condition. 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?

This medicine contains pentraxin-2, a protein that activates certain cells of the immune system (the body's natural defences) that are involved in breaking down and removing 'debris' material from the body. Pentraxin-2 is expected to help these immune cells recognise the debris that causes inflammation and fibrosis in the lungs of patients with idiopathic pulmonary fibrosis. It is thought to do so by attaching to certain molecules (called DAMP) on the surface of dead cells so that they can be recognised by immune cells as debris. Pentraxin-2 is also expected to increase production of other immune cells that produce the anti-inflammatory and anti-fibrotic protein IL-10.This is expected to stop the accumulation of deposits in the lungs, reduce the inflammation and help to relieve the symptoms of idiopathic pulmonary fibrosis.

Pentraxin-2 is made by a method known as 'recombinant DNA technology': it is made by a cell that has received a gene (DNA) that makes the cell able to produce it.

What is the stage of development of this medicine?

The effects of recombinant human pentraxin-2 have been evaluated in experimental models.

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

At the time of submission, recombinant human pentraxin-2 was not authorised anywhere in the EU for idiopathic pulmonary fibrosis. Orphan designation of the medicine 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 13 June 2012 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 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	Recombinant human pentraxin-2	Treatment of idiopathic pulmonary fibrosis
Bulgarian	Рекомбинантен човешки пентраксин-2	Лечение на идиопатична белодробна фиброза
Croatian	Rekombinantni ljudski pentraksin-2	Liječenje idiopatske plućne fibroze
Czech	Rekombinantní humánní pentraxin-2	Léčba idiopatické plicní fibrózy
Danish	Rekombinant human pentraxin-2	Behandling af idiopatisk lungefibrose
Dutch	Recombinant humaan pentraxine-2	Behandeling van idiopathische longfibrose
Estonian	Rekombinantne inimese pentraksiin-2	Idiopaatilise kopsufibroosi ravi
Finnish	Geeniteknisesti tuotettu ihmisen pentraksiini-2	Idiopaattisen keuhkofibroosin hoito
French	Pentraxine-2 humaine recombinante	Traitement de la fibrose pulmonaire idiopathique
German	Rekombinantes humanes Pentraxin-2	Behandlung von Idiopathischer Pulmonaler Fibrose
Greek	Ανασυνδυασμένη ανθρώπινη πεντραξίνη-2	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Rekombináns humán pentraxin-2	Idiopathiás tüdőfibrózis kezelése
Italian	Pentraxina-2 ricombinante umana	Trattamento della fibrosi polmonare idiopatica
Latvian	Rekombinantais cilvēka pentraksīns 2	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	Rekombinantinis žmogaus pentraksinas-2	Idiopatinės plaučių fibrozės gydymas
Maltese	Pentraxin-2 rikombinanti uman	Kura tal-fibrożi pulmonari idjopatika
Polish	Rekombinowana ludzka pentraksyna 2	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Pentraxina-2 humana recombinante	Tratamento da fibrose pulmonar idiopática
Romanian	Pentraxina-2 umană recombinantă	Tratamentul fibrozei pulmonare idiopatice
Slovak	Rekombinantný ľudský pentraxín-2	Liečba idiopatickej pľúcnej fibrózy
Slovenian	Rekombinantni humani pentraksin-2	Zdravljenje idiopatske pljučne fibroze
Spanish	Pentraxina-2 humana recombinante	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Rekombinant humant pentraxin-2	Behandling av idiopatisk lungfibros
Norwegian	Rekombinant humant pentraksin-2	Behandling av idiopatisk lungefibrose
Icelandic	Raðbrigða manna pentraxín-2	Meðferð sjálfvakinnar bandvefsmyndunar í lungum

[1] At the time of transfer of sponsorship