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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tralokinumab for the treatment of idiopathic pulmonary fibrosis

On 8 November 2012, orphan designation (EU/3/12/1061) was granted by the European Commission to MedImmune Ltd, United Kingdom, for tralokinumab 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 deposition of collagen and fibrous tissue in the lungs. This causes the lung tissue to become inflamed and thick and to 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 3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of not more than 152,000 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?

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 tralokinumab might be of significant benefit for patients with idiopathic pulmonary fibrosis because early studies in experimental models

[*]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 506,300,000 (Eurostat 2011).

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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 tralokinumab, a substance that attaches to a chemical messenger in the body called interleukin-13. Interleukin-13 is present in increased amounts in the lungs of people with idiopathic pulmonary fibrosis, and seems to play a role in the development of fibrous tissue and scarring of the lungs in this condition. By attaching to the excess interleukin-13 and preventing it from working, tralokinumab is expected to reduce this process and slow down the decline in the ability of the lungs to work properly.

What is the stage of development of this medicine?

The effects of tralokinumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with tralokinumab in patients with idiopathic pulmonary fibrosis had been started.

At the time of submission, tralokinumab was not authorised anywhere in the EU for idiopathic pulmonary fibrosis. Orphan designation of tralokinumab has 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 October 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 substance	Indication
English	Tralokinumab	Treatment of idiopathic pulmonary fibrosis
Bulgarian	Тралокинумаб	Лечение на идиопатична белодробна фиброза
Czech	Tralokinumab	Léčba idiopatické plicní fibrózy
Danish	Tralokinumab	Behandling af idiopatisk lungefibrose
Dutch	Tralokinumab	Behandeling van idiopathische longfibrose
Estonian	Tralokinumaab	Idiopaatilise kopsufibroosi ravi
Finnish	Tralokinumabi	Idiopaattisen keuhkofibroosin hoito
French	Tralokinumab	Traitement de la fibrose pulmonaire idiopathique
German	Tralokinumab	Behandlung von Idiopathischer Pulmonaler Fibrose
Greek	Τραλοκινουμάμπη	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Tralokinumab	Idiopathiás tüdőfibrózis kezelése
Italian	Tralokinumab	Trattamento della fibrosi polmonare idiopatica
Latvian	Tralokinumabs	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	Tralokinumabas	Idiopatinės plaučių fibrozės gydymas
Maltese	Tralokinumab	Kura tal-fibrożi pulmonari idjopatika
Polish	Tralokinumab	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Tralokinumab	Tratamento da fibrose pulmonar idiopática
Romanian	Tralokinumab	Tratamentul fibrozei pulmonare idiopatice
Slovak	Tralokinumab	Liečba idiopatickej pľúcnej fibrózy
Slovenian	Tralokinumab	Zdravljenje idiopatske pljučne fibroze
Spanish	Tralokinumab	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Tralokinumab	Behandling av idiopatisk lungfibros
Norwegian	Tralokinumab	Behandling av idiopatisk lungefibrose
Icelandic	Tralokinumab	Meðferð sjálfvakinnar bandvefsmyndunar í lungum

[1] At the time of designation