27 August 2014
EMA/COMP/374511/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised anti-alpha v beta 6 monoclonal antibody for the treatment of idiopathic pulmonary fibrosis

On 29 July 2014, orphan designation (EU/3/14/1301) was granted by the European Commission to Biogen Idec Ltd, United Kingdom, for humanised anti-alpha v beta 6 monoclonal antibody 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 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 was equivalent to a total of not more than 153,000 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 mild to moderate idiopathic pulmonary fibrosis.

The sponsor has provided sufficient information to show that humanised anti-alpha v beta 6 monoclonal antibody might be of significant benefit to patients with idiopathic pulmonary fibrosis based

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

on results from early studies showing a reduction in fibrosis and because the medicine works in a different way to pirfenidone, specifically targeting 'alpha v beta 6', a protein involved in the formation of fibrous tissue. These assumptions 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 is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific target. The target for this medicine is a protein called alpha v beta 6, which is found in high amounts in the lungs of patients with idiopathic pulmonary fibrosis, where it plays a role in the formation of fibrous tissue.

By attaching to alpha v beta 6 and blocking its actions, the medicine is expected to reduce the formation of fibrous tissue in the lungs and thereby improve the patient's symptoms.

What is the stage of development of this medicine?

The effects of the medicine 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, the medicine was not authorised anywhere in the EU for idiopathic pulmonary fibrosis. Orphan designation had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 June 2014 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:

Biogen Idec Limited
Innovation House
70 Norden Road
Maidenhead
Berkshire SL6 4AY
United Kingdom
Tel. +44 162 8501 000
Fax +44 162 8501 010
E-mail: ukreception@biogenidec.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	Humanised anti-alpha v beta 6 monoclonal antibody	Treatment of idiopathic pulmonary fibrosis
Bulgarian	Хуманизирано анти-алфа срещу бета 6 моноклонално антитяло	Лечение на идиопатична белодробна фиброза
Croatian	Humanizirano anti-alfa v beta 6 monoklonsko protutijelo	Liječenju idiopatske plućne fibroze
Czech	Humanizované anti-alfa v beta 6 monoklonální protilátka	Léčba idiopatické plicní fibrózy
Danish	Humaniseret anti-alpha v beta 6 monoklonalt antistof	Behandling af idiopatisk lungefibrose
Dutch	Gehumaniseerd anti-alpha v beta 6 monoklonaal antilichaam	Behandeling van idiopathische longfibrose
Estonian	Humaniseeritud anti-alfa v beeta 6 monoklonaalne antikeha	Idiopaatilise kopsufibroosi ravi
Finnish	Humanisoitu anti-alfa v beeta 6 monoklonaalinen vasta-aine	Idiopaattisen keuhkofibroosin hoito
French	Anticorps monoclonal humanisé anti-alpha v béta 6	Traitement de la fibrose pulmonaire idiopathique
German	Humanisierter anti-alpha v beta 6 monoklonaler Antikörper	Behandlung von idiopathischer pulmonaler Fibrose
Greek	Ανθρώποποιημένο αντι-αvβ6 μονοκλωνικό αντίσωμα	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Humanizált anti-alfa-béta v 6 monoklonális antitest	Idiopathiás tüdőfibrózis kezelése
Italian	anticorpo monoclonale umanizzato anti-alfa v beta 6	Trattamento della fibrosi polmonare idiopatica
Latvian	Humanizēta anti-alfa-V beta 6 monoklonāla antiviela	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	Humanizuotas anti-alfa prieš beta 6 monokloninis antikūnas	Idiopatinės plaučių fibrozės gydymas
Maltese	Antikorp monoklonali umanizzat kontra alfa v beta 6	Kura tal-fibrożi pulmonari idjopatika
Polish	Humanizowane anty-alfa v beta 6 przeciwciało monoklonalne	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Anticorpo monoclonal humanizado anti-alpha v beta 6	Tratamento da fibrose pulmonar idiopática
Romanian	Anticorp monoclonal umanizat anti-alfa v beta 6	Tratamentul fibrozei pulmonare idiopatice
Slovak	Humanizovaná anti-alfa v beta 6 monoklonálna protilátka	Liečba idiopatickej pľúcnej fibrózy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humanizirano anti-alfa v beta 6 monoklonsko protitelo	Zdravljenje idiopatske pljučne fibroze
Spanish	Anti-alfa v beta 6 anticuerpo monoclonal humanizado	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Humaniserad anti-alfa v beta 6 monoklonal antikropp	Behandling av idiopatisk lungfibros
Norwegian	Humanisert anti-alfa v beta 6 monoklonalt antistoff	Behandling av idiopatisk lungefibrose
Icelandic	Mannaaðlagað anti-alfa v beta 6 einstofna mótefni	Meðferð sjálfvakinnar bandvefsmyndunar í lungum