12 January 2015
EMA/COMP/646696/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

1-(6-Benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid for the treatment of idiopathic pulmonary fibrosis

On 19 November 2014, orphan designation (EU/3/14/1362) was granted by the European Commission to Inventiva, France, for 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid 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 the medicine might be of significant benefit for patients with idiopathic pulmonary fibrosis because results of studies in experimental

*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

models show that the medicine may reduce fibrosis (the abnormal growth of connective tissue). 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 is a chemical substance that is expected to work by activating cells receptors called peroxisome proliferator-activated receptors (PPARs). PPARs are thought to regulate fibrosis. By activating PPARs, this medicine is expected to reduce the fibrosis seen in idiopathic pulmonary fibrosis, thereby relieving the symptoms of this condition.

What is the stage of development of this medicine?

The effects of 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for idiopathic pulmonary fibrosis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 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:

Inventiva
50 route de Dijon
21121 Daix
France
Tel. +33 3 80 44 75 00
Fax +33 3 80 44 75 61
E-mail: info@inventivapharma.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	1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid	Treatment of idiopathic pulmonary fibrosis
Bulgarian	1-(6-бензотиазолилсулфонил)-5-хлоро-1H-индол-2-бутанова киселина	Лечение на идиопатична белодробна фиброза
Croatian	1-(6-benzotiazolilsulfonil)-5-kloro-1H-indol-2-butanoatna kiselina	Liječenje idiopatske plućne fibroze
Czech	1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indol-2-kyselina máselná	Léčba idiopatické plicní fibrózy
Danish	1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indol-2-butansyre	Behandling af idiopatisk lungefibrose
Dutch	1-(6-benzothiazolylsulfonyl)-5-chloor-1H-indool-2-butaanzuur	Behandeling van idiopathische longfibrose
Estonian	1-(6-bensotiasoolüülsulfonüül)-5-kloro-1H-indool-2-butanoonhape	Idiopaatilise kopsufibroosi ravi
Finnish	1-(6-bentsotiatsolyylisulfonyyli)-5-kloori-1H-indoli-2-butaanihappo	Idiopaattisen keuhkofibroosin hoito
French	1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-acide butanoïque	Traitement de la fibrose pulmonaire idiopathique
German	1-(6-Benzothiazolylsulfonyl)-5-chlor-1H-indol-2-butansäure	Behandlung von idiopathischer pulmonaler Fibrose
Greek	1-(6-βενζοθειαζολυλσουλφονυλ)-5-χλορο-1H-*ινδολο*-2-βουτανοϊκό οξύ	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	1-(6-benzo-tiazolil-szulfonil)-5-klóro-1H-indol-2-vajsav	Idiopathiás tüdőfibrózis kezelése
Italian	acido 1-(6-benzotiazolilsulfonil)-5-cloro-1H-indolo-2-butanoico	Trattamento della fibrosi polmonare idiopatica
Latvian	1-(6-benzotiazolilsulfonil)-5-hloro-1H-indola-2-sviestskābe	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	1-(6-benzotiazolilsulfonil)-5-chloro-1H-indolo-2-butano rūgštis	Idiopatinės plaučių fibrozės gydymas
Maltese	1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid	Kura tal-fibroži pulmonari idjopatika
Polish	Kwas 1-(6-benzotiazolilosulfonylo)-5-chloro-1H-indolo-2-butanowy	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Ácido 1-(6-benzotiazolilsulfonil)-5-cloro-1H-indol-2-butanóico	Tratamento da fibrose pulmonar idiopática
Romanian	acid 1-(6-benzotiazolilsulfonil)-5-cloro-1H-indol-2-butanoic	Tratamentul fibrozei pulmonare idiopatice
Slovak	Kyselina 1-(6-benzotiazolylsulfonyl)-5-chlór-1H-indol-2-butánová	Liečba idiopatickej pľúcnej fibrózy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	1-(6-benzotiazolilsulfonil)-5-kloro-1H-indol-2-butanojska kislina	Zdravljenje idiopatske pljučne fibroze
Spanish	Ácido 1-(6-benzotiazolilsulfonil)-5-cloro-1H-indol-2-butanoico	Tratamiento de la fibrosis pulmonar idiopática
Swedish	1-(6-benzotiazolylsulfonyl)-5-kloro-1H-indol-2-butansyra	Behandling av idiopatisk lungfibros
Norwegian	1-(6-benzotiazolylsulfonyl)-5-klor-1H-indol-2-butansyre	Behandling av idiopatisk lungefibrose
Icelandic	1-(6-benzótíazólýlsúlfónýl)-5-klóró-1H-indól-2-smjörsýra	Meðferð sjálfvakinnar bandvefsmyndunar í lungum