5 September 2016
EMA/COMP/446359/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt for the treatment of idiopathic pulmonary fibrosis

On 14 July 2016, orphan designation (EU/3/16/1692) was granted by the European Commission to Vicore Pharma AB, Sweden, for 3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt 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 long-term debilitating and life-threatening 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 approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 180,000 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) and Ofev (nintedanib) were authorised in the EU to treat idiopathic pulmonary fibrosis.

[*]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 513,700,000 (Eurostat 2016).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with idiopathic pulmonary fibrosis because early studies in the laboratory show that it reduces pulmonary hypertension (high blood pressure in the blood vessels of the lungs, which is a common complication of the condition) as well as lung fibrosis (scarring). 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 stimulates a receptor called the angiotensin II type 2 receptor (AT2R). This receptor is found at high levels in damaged tissue, where it is thought to be involved in protecting tissues. Exactly how the medicine works in the condition is not clear, but it is believed that by attaching to AT2R, it reduces the production of inflammatory substances and the development of hard, fibrous tissue in the lungs, including in the blood vessels, thus controlling symptoms of the disease.

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, no clinical trials with this 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 16 June 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt	Treatment of idiopathic pulmonary fibrosis
Bulgarian	3-[4-(1H-имидазол-1-илметил)фенил]-5-(2-метилпропил)тиофен-2-[(N-бутилоксилкарбамат)-сулфонамид] натриева сол	Лечение на идиопатична белодробна фиброза
Croatian	3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofen-2-[(N-butiloksilkarbamat)-sulfonamid] natrijeva sol	Liječenje idiopatske plućne fibroze
Czech	Butyl-({3-[4-(1H-imidazol-1-ylmethyl)fenyl]-5-isobutyl-2-thienyl}sulfonyl)karbamát, sodná sůl	Léčba idiopatické plicní fibrózy
Danish	3-[4-(1H-imidazol-1-yl-metyl)phenyl]-5-(2-metylpropyl)thiophen-2-[(N-butyloxylcarbamat)-sulfonamid] natriumsalt	Behandling af idiopatisk lungefibrose
Dutch	3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiofeen-2-[(N-butyl oxy carbamate)-sulfonamide] sodium zout	Behandeling van idiopathische longfibrose
Estonian	3-[4-(1H-imidasool-1-üülmetüül)fenüül]-5-(2-metüülpropüül)tiofeen-2-[(N-butüüloksüülkarbamaat)-sulfoonamiid] naatriumsool	Idiopaatilise kopsufibroosi ravi
Finnish	3-[4-(1H-imidatsoli-1-yylimetyyli)fenyyli]-5-(2-metyylipropyyli)tiofeeni-2-[(N-butyylioksyylikarbamaatti)-sulfonamidi]-natriumsuola	Idiopaattisen keuhkofibroosin hoito
French	3-[4-(1H-imidazole-1-ylméthyl)phényle]-5-(2-méthylpropyle)thiophène-2-[(N-butyloxylcarbamate)-sulfonamide] sel de sodium	Traitement de la fibrose pulmonaire idiopathique
German	3-[4-(1H-Imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophen-2-[(N-butyloxylcarbamat)-sulfonamid] Natriumsalz	Behandlung von idiopathischer pulmonaler Fibrose
Greek	Μετά νατρίου άλας του 3-[4-(1H-ιμιδαζολ-1-υλ-μεθυλο)φαινυλο]-5-(2-μεθυλοπροπυλο)θειοφαινο-2-[(N-βουτυλοξυκαρβαμιδιο)-σουλφοναμιδίου]	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofén-2-[(N-butiloxikarbonil)-szulfonamid] nátriumsó	Idiopathiás tüdőfibrózis kezelése
Italian	Sale sodico, 3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofene-2-[(N-butiloxilcarbamato)-sulfonamide]	Trattamento della fibrosi polmonare idiopatica
Latvian	3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofēn-2-[(N-butiloksilkarbamāt)-sulfonamīda] nātrija sāls	Idiopātiskās plaušu fibrozes ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofen-2-[(N-butiloksilkarbamato)-sulfonamido] natrio druska	Idiopatinės plaučių fibrozės gydymas
Maltese	3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] melħ tas-sodium	Kura tal-fibrożi pulmonari idjopatika
Polish	sól sodowa 3-[4-(1H-imidazol-1-ilometylo)fenylo]-5-(2-metylopropylo)tiofeno-2-[(N-butyksykarbaminiano)-sulfonamidu]	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Sal sódico 3-[4-(1*H*-imidazol-1-ilmetilo)fenilol]-5-(2-metilpropil)tiofeno-2-[(*N*-butiloxilocarbamato)-sulfanamida]	Tratamento da fibrose pulmonar idiopática
Romanian	3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofen-2-[(N-butiloxilcarbamat)-sulfamidă] sare de sodiu	Tratamentul fibrozei pulmonare idiopatice
Slovak	Sodná soľ 3-[4-(1H-imidazol-1-ylmetyl)fenyl]-5-(2-metylpropyl)tiofén-2-[(N-butyloxylkarbamát)-sulfonamidu]	Liečba idiopatickej pľúcnej fibrózy
Slovenian	3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofen-2-[(N-butiloksikarbamat)-sulfonamid] natrijeva sol	Zdravljenje idiopatske pljučne fibroze
Spanish	Sal sódica, 3-[4-(1H-imidazol-1-ilmetil)fenil]-5-(2-metilpropil)tiofeno-2-[(N-butiloxilcarbamato)-sulfonamida]	Tratamiento de la fibrosis pulmonar idiopática
Swedish	3-[4-(1H-imidazol-1-ylmetyl)fenyl]-5-(2-metylpropyl)tiofen-2-[(N-butyloxylkarbamat)-sulfonamid] natriumsalt	Behandling av idiopatisk lungfibros
Norwegian	3-[4-(1H-imidazol-1-ylmetyl)fenyl]-5-(2-metylpropyl)tiofen-2-[(N-butyloksylkarbamat)-sulfonamid] natriumsalt	Behandling av idiopatisk lungefibrose
Icelandic	3-[4-(1H-ímídasól-1-ýlmetýl)fenýl]-5-(2-metýlprópýl)tíófen-2-[(N-bútýloxýlkarbamat)-súlfónamíð] natríumsalt	Meðferð sjálfvakinnar bandvefsmyndunar í lungum