22 November 2018
EMA/521616/2018

Public summary of opinion on orphan designation

(S)-(−)-3-(4-aminophenyl)-2-methoxypropanoic acid for the treatment of idiopathic pulmonary fibrosis

On 24 August 2018, orphan designation (EU/3/18/2056) was granted by the European Commission to Nogra Pharma Limited, Ireland, for (S)-(−)-3-(4-aminophenyl)-2-methoxypropanoic acid (also known as GED-0507-34-Levo) 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 no longer work normally, reducing the amount of oxygen that gets 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 155,000 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.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with idiopathic pulmonary fibrosis because laboratory studies suggested that it

*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 517,400,000 (Eurostat 2018).

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

An agency of the European Union

© European Medicines Agency, 2018. Reproduction is authorised provided the source is acknowledged.

might work better than authorised medicines. 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?

The medicine is expected to activate targets inside the cell called PPAR-gamma receptors. These receptors control various processes in the body that reduce inflammation and production of fibrous tissue. By activating PPAR-gamma receptors in the airways, the medicine is expected to reduce inflammation and the development of fibrosis in the lungs and so control the 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 the medicine in patients with idiopathic pulmonary fibrosis had been started.

At the time of submission, this 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 19 July 2018 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:

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	(S)-(−)-3-(4-aminophenyl)-2-methoxypropanoic acid	Treatment of idiopathic pulmonary fibrosis
Bulgarian	(S)-(−)-3-(4-аминофенил)-2-метоксипропанова киселина	Лечение на идиопатична белодробна фиброза
Croatian	(S)-(−)-3-(4-aminofenil)-2-metoksi propionska kiselina	Liječenje idiopatske plućne fibroze
Czech	(S)-(−)-3-(4-aminofenyl)-2-methoxypropionová kyselina	Léčba idiopatické plicní fibrózy
Danish	(S)-(−)-3-(4-aminophenyl)-2-methoxypropionsyre	Behandling af idiopatisk lungefibrose
Dutch	(S)-(−)-3-(4-aminofenyl)-2-methoxypropaanzuur	Behandeling van idiopathische longfibrose
Estonian	(S)-(−)-3-(4-aminofenüül)-2-metoksüpropioonhape	Idiopaatilise kopsufibroosi ravi
Finnish	(S)-(−)-3-(4-aminofenyyli)-2-metoksipropionihappo	Idiopaattisen keuhkofibroosin hoito
French	Acide (S)-(−)-3-(4-aminophényl)-2-méthoxypropanoïque	Traitement de la fibrose pulmonaire idiopathique
German	(S)-(−)-3-(4-Aminophenyl)-2-methoxypropansäure	Behandlung von idiopathischer pulmonaler Fibrose
Greek	(S)-(−)-3-(4-αμινοφαινυλ)-2-μεθοξυπροπανοϊκό οξύ	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	(S)-(−)-3-(4-aminofenil)-2-metoxipropionsav	Idiopathiás tüdőfibrózis kezelése
Italian	Acido (S)-(−)-3-(4-aminofenil)-2-metossipropanoico	Trattamento della fibrosi polmonare idiopatica
Latvian	(S)-(−)-3-(4-aminofenil)-2-metoksipropānskābe	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	(S)-(−)-3-(4-aminofenil)-2-metoksipropanoinė rūgštis	Idiopatinės plaučių fibrozės gydymas
Maltese	Aċidu (S)-(−)-3-(4-aminofenil)-2-metossipropanojiku	Kura tal-fibrożi pulmonari idjopatika
Polish	Kwas metoksy(S)-(−)-3-(4-aminofenylo)-2-propionowy	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Ácido (S)-(−)-3-(4-aminofenil)-2-metoxipropanóico	Tratamento da fibrose pulmonar idiopática
Romanian	Acid (S)-(−)-3-(4-aminofenil)-2-metoxipropanoic	Tratamentul fibrozei pulmonare idiopatice
Slovak	Kyselina (S)-(−)-3-(4-aminofenyl)-2-metoxypropánová	Liečba idiopatickej pľúcnej fibrózy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	(S)-(-)-3-(4-aminofenil)-2-metoksi-propanojska kislina	Zdravljenje idiopatske pljučne fibroze
Spanish	Ácido (S)-(-)-3-(4-aminofenil)-2-metoxipropanoico	Tratamiento de la fibrosis pulmonar idiopática
Swedish	(S)-(-)-3-(4- aminofenyl)-2-metoxipropansyra	Behandling av idiopatisk lungfibros
Norwegian	(S)-(-)-3-(4-aminofenyl)-2-metoksypropansyre	Behandling av idiopatisk lungefibrose
Icelandic	(S)-(−)-3-(4-amínófenýl)-2-metoxýprópansýra	Meðferð sjálfvakinnar bandvefsmyndunar í lungum