



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Tilorone for the treatment of idiopathic pulmonary fibrosis

On 24 August 2018, orphan designation (EU/3/18/2069) was granted by the European Commission to Professor Marjukka Myllärniemi, Finland, for tilorone 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 was as effective as the authorised treatments and could reduce development of fibrous tissue when given by inhalation. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

*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).



How is this medicine expected to work?

The exact way that tilorone works in this condition is not fully understood, but it is thought to act in several complementary ways. These include blocking the action of a substance called gremlin-1, which is present at high levels in the lungs of patients with idiopathic pulmonary fibrosis. Gremlin-1 stimulates the development of fibrous tissue by interfering with protective molecules called bone morphogenetic proteins (BMP). In addition, tilorone is thought to reduce the activity of another substance, TGF-beta, that is implicated in the development of fibrous tissue. Together these actions are expected to slow down the progression of idiopathic pulmonary fibrosis.

What is the stage of development of this medicine?

The effects of tilorone have been evaluated in experimental models.

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

At the time of submission, the medicine was authorised in Russia and some other non-EU countries for viral infections.

At the time of submission, tilorone 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Tilorone	Treatment of idiopathic pulmonary fibrosis
Bulgarian	Тилорон	Лечение на идиопатична белодробна фиброза
Croatian	Tiloron	Liječenje idiopatske plućne fibroze
Czech	Tilorone	Léčba idiopatické plicní fibrózy
Danish	Tilorone	Behandling af idiopatisk lungefibrose
Dutch	Tilorone	Behandeling van idiopathische longfibrose
Estonian	Tilooron	Idiopaatilise kopsufibroosi ravi
Finnish	Tiloroni	Idiopaattisen keuhkofibroosin hoito
French	Tilorone	Traitement de la fibrose pulmonaire idiopathique
German	Tiloron	Behandlung von idiopathischer pulmonaler Fibrose
Greek	Τιλορόνη	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Tilorone	Idiopathiás tüdőfibrózis kezelése
Italian	Tilorone	Trattamento della fibrosi polmonare idiopatica
Latvian	Tilorons	Idiopātiskās plaušu fibrozēs ārstēšana
Lithuanian	Tiloronas	Idiopatinės plaučių fibrozės gydymas
Maltese	Tiloron	Kura tal-fibrozi pulmonari idjopatika
Polish	Tilorone	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Tilorona	Tratamento da fibrose pulmonar idiopática
Romanian	Tiloron	Tratamentul fibrozei pulmonare idiopatică
Slovak	Tilorone	Liečba idiopatickej pľúcnej fibrózy
Slovenian	Tiloron	Zdravljenje idiopatske pljučne fibroze
Spanish	Tilorona	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Tilorone	Behandling av idiopatisk lungfibros
Norwegian	Tilorone	Behandling av idiopatisk lungefibrose
Icelandic	Tiloron	Meðferð sjálfvakinnar bandvefsmyndunar í lungum

¹ At the time of designation