



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Sodium cromoglicate for the treatment of idiopathic pulmonary fibrosis

On 26 June 2020, orphan designation EU/3/20/2294 was granted by the European Commission to IQVIA RDS Spain S.L., Spain, for sodium cromoglicate for the treatment of idiopathic pulmonary fibrosis.

What is idiopathic pulmonary fibrosis?

Idiopathic pulmonary fibrosis is a disease characterised by progressive deposition of collagen and fibrous tissue in the lungs. This causes the lung tissue to become thick and 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 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of 166,000 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 early data suggested that sodium cromoglicate may reduce cough when given to patients already receiving either pirfenidone or

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).



nintedanib. 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?

Idiopathic pulmonary fibrosis is thought to be caused by excess activity of fibroblasts, a type of skin cell that produces collagen and other skin components. Sodium cromoglycate is expected to act on cells of the immune system (the body's natural defences) called mast cells and prevent them from stimulating fibroblasts. When taken directly into the lungs using a nebulizer (a machine that changes a solution into an aerosol that the patient can breathe in), sodium cromoglycate is expected to prevent the build-up of fibrous tissue and improve symptoms of the disease.

What is the stage of development of this medicine?

The effects of sodium cromoglycate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with sodium cromoglycate in patients with idiopathic pulmonary fibrosis were ongoing.

At the time of submission, sodium cromoglycate was not authorised anywhere in the EU for the treatment of idiopathic pulmonary fibrosis. Orphan designation of sodium cromoglycate had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 May 2020, 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](#).

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.

Withdrawn

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sodium cromoglicate	Treatment of idiopathic pulmonary fibrosis
Bulgarian	Натриев кромогликат	Лечение на идиопатична белодробна фиброза
Croatian	Natrijev kromoglikat	Liječenje idiopatske plućne fibroze
Czech	Kromoglykát sodný	Léčba idiopatické plicní fibrózy
Danish	Natriumcromoglicat	Behandling af idiopatisk lungefibrose
Dutch	Natriumcromoglicaat	Behandeling van idiopathische longfibrose
Estonian	Naatriumkromoglükaat	Idiopaatilise kopsufibroosi ravi
Finnish	Natriumkromoglikaatti	Idiopaattisen keuhkofibroosin hoito
French	Cromoglicat de sodium	Traitement de la fibrose pulmonaire idiopathique
German	Natriumcromoglicat	Behandlung von idiopathischer pulmonaler Fibrose
Greek	Χρωμογλυκικό νάτριο	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Nátrium-kromoglikát	Idiopathiás tüdőfibrózis kezelése
Italian	Sodio cromoglicato	Trattamento della fibrosi polmonare idiopatica
Latvian	Nātrijs kromoglikāts	Idiopātiskās plaušu fibrozēs ārstēšana
Lithuanian	Natrio kromoglikatas	Idiopatinės plaučių fibrozės gydymas
Maltese	Kromoglikat tas-sodju	Kura tal-fibrozi pulmonari idjopatika
Polish	Kromoglikan sodu	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Cromoglicato de sódio	Tratamento da fibrose pulmonar idiopática
Romanian	Cromoglicat de sodium	Tratamentul fibrozei pulmonare idiopatice
Slovak	Kromoglykát sodný	Liečba idiopatickej pľúcnej fibrózy
Slovenian	Natrijev kromoglikat sodný	Zdravljenje idiopatske pljučne fibroze
Spanish	Cromoglicato de sodio	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Natriumkromoglikat	Behandling av idiopatisk lungfibros
Norwegian	Natriumkromoglikat	Behandling av idiopatisk lungefibrose
Icelandic	Nátríumkróomóglíkat	Meðferð sjálfvakinnar bandvefsmyndunar í lungum

¹ At the time of designation