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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

3-Pentylbenzeneacetic acid sodium salt for the treatment of idiopathic pulmonary fibrosis

On 9 October 2015, orphan designation (EU/3/15/1550) was granted by the European Commission to ProMetic BioTherapeutics Ltd, United Kingdom, for 3-pentylbenzeneacetic acid 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 not more than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 154,000 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. Early studies showed 3-pentylbenzeneacetic acid

*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 512,900,000 (Eurostat 2015).



sodium salt to have more effect in reducing fibrosis than currently authorised products. Its effects were further increased when used in combination with currently authorised products. 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 works by attaching to two receptors that are found in different types of white blood cells and are thought to be involved in inflammation and the development of fibrosis. By attaching to these receptors, the medicine is expected to help reduce inflammation and fibrosis, thereby relieving symptoms of patients with idiopathic pulmonary fibrosis.

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, the medicine was not authorised anywhere in the EU for idiopathic pulmonary fibrosis. Orphan designation had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 September 2015 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

For details of the current sponsor of the orphan designation please refer to the information on the main web page of this Public Summary of Opinion.

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	3-pentylbenzeneacetic acid sodium salt	Treatment of idiopathic pulmonary fibrosis
Bulgarian	Натриева сол на 3-пентилбензеноцетна киселина	Лечение на идиопатична белодробна фиброза
Croatian	Natrijev 3-pentilfenilacetat	Liječenje idiopatske plućne fibroze
Czech	sodná sůl kyseliny 3-pentylbenzenoctové	Léčba idiopatické plicní fibrózy
Danish	3-pentylbenzenacetylsyre natriumsalt	Behandling af idiopatisk lungefibrose
Dutch	3-pentylbenzeenazijnzuur natriumzout	Behandeling van idiopathische longfibrose
Estonian	3-pentüülbenseenäädikhappe naatriumisool	Idiopaatilise kopsufibroosi ravi
Finnish	3-pentyyli-fenyylietikkahapon natriumsuola	Idiopaattisen keuhkofibroosin hoito
French	Sel sodique d'acide 3-pentylbenzènacétique	Traitement de la fibrose pulmonaire idiopathique
German	3-Pentylbenzoesigsäure-Natriumsalz	Behandlung von idiopathischer pulmonaler Fibrose
Greek	Άλας του 3-πεντυλβενζολοξικού οξέος με νάτριο	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	3-pentyl-benzene ecetsav nátriumsó	Idiopathiás tüdőfibrózis kezelése
Italian	Acido 3-pentilbenzenacetico, sale sodico	Trattamento della fibrosi polmonare idiopatica
Latvian	3-pentilfeniletikskābes nātrija sāls	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	3-pentilbenzeno acto rūgšties natrio druska	Idiopatinės plaučių fibrozės gydymas
Maltese	3-pentylbenzeneacetic acid sodium salt	Kura tal-fibrozi pulmonari idjopatika
Polish	Sól sodowa kwasu 3-pentylbenzenoocetowego	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Sal sódico de ácido 3-pentilbenzenoacético	Tratamento da fibrose pulmonar idiopática
Romanian	Sare de sodiu a acidului 3-pentilbenzenacetic	Tratamentul fibrozei pulmonare idiopatice
Slovak	Sodná soľ kyseliny 3-pentylbenzènoctovej	Liečba idiopatickej pľúcnej fibrózy
Slovenian	3-pentil-benzenocetna kislina, natrijeva sol	Zdravljenje idiopatske pljučne fibroze
Spanish	Ácido 3-pentilfenilacético, sal sódica	Tratamiento de la fibrosis pulmonar idiopática
Swedish	3-pentylbensenättiksyra, natriumsalt	Behandling av idiopatisk lungfibros
Norwegian	3-pentylbenzen eddiksyre natriumsalt	Behandling av idiopatisk lungefibrose
Icelandic	3-pentýlbensenediksyra, natríumsalt	Meðferð sjálfvakinnar bandvefsmýndunar í lungum

¹ At the time of designation