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## Public summary of opinion on orphan designation

### Dimethyl fumarate for the treatment of Friedreich's ataxia

On 21 March 2018, orphan designation (EU/3/18/1990) was granted by the European Commission to PharmaBio Consulting, Germany, for dimethyl fumarate for the treatment of Friedreich's ataxia.

#### What is Friedreich's ataxia?

Friedreich's ataxia is an inherited disease that causes a range of symptoms that worsen over time, including difficulty walking, inability to co-ordinate movements, muscle weakness, speech problems, damage to the heart muscle, and diabetes.

Patients with Friedreich's ataxia do not have enough frataxin, a protein that regulates iron in mitochondria (energy-producing components of cells). As a result, iron builds up within the cells, which in turn results in the production of toxic forms of oxygen that damage cells in the brain, the spinal cord and nerves, as well as in the heart and pancreas.

Friedreich's ataxia is a debilitating and life-threatening disease because of the worsening of symptoms over time. The disease is usually fatal in early adulthood.

#### What is the estimated number of patients affected by the condition?

At the time of designation, Friedreich's ataxia affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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, no satisfactory methods were authorised in the EU for the treatment of Friedreich's ataxia. Different treatments were used to relieve the symptoms of the disease, such as medicines for diabetes and heart problems. Patients were also offered walking aids to allow them to remain as independent as possible, and other devices to assist them with everyday tasks such as eating and taking care of themselves. Speech therapy and physiotherapy were also used.

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\*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 medicine is involved in the activation of a protein called Nrf2, which can reduce inflammation and protect cells against toxic forms of oxygen. In addition, dimethyl fumarate is also involved in the development of mitochondria in the cells and seems to increase levels of frataxin. These effects are expected to reduce 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 Friedreich's ataxia had been started.

Dimethyl fumarate is authorised in the EU for the treatment of psoriasis and multiple sclerosis.

At the time of submission, the medicine was not authorised anywhere in the EU for Friedreich's ataxia. Orphan designation of the medicine had been granted in the USA for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 2018 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Dimethyl fumarate	Treatment of Friedreich's ataxia
Bulgarian	Диметилфумарат	Лечение на атаксия на Фридрайх
Croatian	Dimetil fumarate	Liječenje Friedreichove ataksije
Czech	Dimethyl-fumarát	Léčba Friedrichovy ataxie
Danish	Dimethylfumarat	Behandling af Friedreichs ataksi
Dutch	Dimethylfumaraat	Behandeling van de ataxie van Friedreich
Estonian	Dimetüülfumaraat	Friedreichi ataksia ravi
Finnish	Dimetyylifumaraatti	Friedreichin ataksian hoito
French	Fumarate de diméthyle	Traitement de l'ataxie de Friedreich
German	Dimethylfumarat	Therapie der Friedreichschen Ataxie
Greek	Φουμαρικό διμεθύλιο	Θεραπεία της αταξίας Friedreich
Hungarian	Dimetil-fumarát	Friedreich ataxia kezelése
Italian	Dimetilfumarato	Trattamento dell'ataxia di Friedreich
Latvian	Dimetilfumerāts	Frīdreiha ataksijas ārstēšana
Lithuanian	Dimetilfumaratas	Fridreicho ataksijos gydymas
Maltese	Dimethyl fumarate	Kura tal-atassja ta' Friedreich
Polish	Fumaran dimetylu	Leczenie ataksji Friedreicha
Portuguese	Fumarato de dimetilo	Tratamento da ataxia de Friedreich
Romanian	Dimetil fumarat	Tratamentul ataxiei Friedreich
Slovak	Dimetylfumarát	Liečba Friedreichovej ataxie
Slovenian	Dimetil fumarat	Zdravljenje Friedreichove ataksije
Spanish	Dimetilfumarato	Tratamiento de la ataxia de Friedreich
Swedish	Dimetylfumarat	Behandling av Friedreichs ataxi
Norwegian	Dimetylfumarat	Behandling av Friedreichs ataksi
Icelandic	Dímetyl fúmarat	Meðferð arfgengs mænuslingurs

<sup>1</sup> At the time of designation