



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Leriglitazone for the treatment of Friedreich's ataxia

On 17 October 2019, orphan designation EU/3/19/2212 was granted by the European Commission to Minoryx Therapeutics S.L., Spain, for leriglitazone (also known as MIN-102) 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.

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

At the time of designation, Friedreich's ataxia affected less than 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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

*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 518,400,000 (Eurostat 2019).



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.

How is this medicine expected to work?

The medicine works by activating a receptor (target) on cells called peroxisome proliferator-activated receptor γ (PPAR γ), which is involved in helping mitochondria to work properly. By increasing the activity of PPAR γ , the medicine is expected to improve the function of mitochondria and remove toxic forms of oxygen in patients with Friedreich's ataxia, thereby slowing down the progression of the disease.

What is the stage of development of this medicine?

The effects of leriglitazone have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with leriglitazone in patients with Friedreich's ataxia were ongoing.

At the time of submission, leriglitazone was not authorised anywhere in the EU for the treatment of Friedreich's ataxia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 12 September 2019, 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.

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

Language	Active ingredient	Indication
English	Leriglitzone	Treatment of Friedreich's ataxia
Bulgarian	Лериглитазон	Лечение на атаксия на Фридрайх
Croatian	Leriglitzon	Liječenje Friedreichove ataksije
Czech	Leriglitzon	Léčba Friedrichovy ataxie
Danish	Leriglitzon	Behandling af Friedreichs ataksi
Dutch	Leriglitzone	Behandeling van de ataxie van Friedreich
Estonian	Leriglitasoon	Friedreichi ataksia ravi
Finnish	Leriglitasoni	Friedreichin ataksian hoito
French	Leriglitzone	Traitement de l'ataxie de Friedreich
German	Leriglitzone	Therapie der Friedreichschen Ataxie
Greek	Λεριγλιταζόνη	Θεραπεία της αταξίας Friedreich
Hungarian	Leriglitzon	Friedreich ataxia kezelése
Italian	Leriglitzone	Trattamento dell'ataxia di Friedreich
Latvian	Leriglitzons	Frīdreiha ataksijas ārstēšana
Lithuanian	Leriglitzonas	Friedreicho ataksijos gydymas
Maltese	Leriglitażon	Kura tal-atassja ta' Friedreich
Polish	Leriglitzon	Leczenie ataksji Friedreicha
Portuguese	Leriglitzona	Tratamento da ataxia de Friedreich
Romanian	Leriglitzonă	Tratamentul ataxiei Friedreich
Slovak	Leriglitzón	Liečba Friedreichovej ataxie
Slovenian	Leriglitzon	Zdravljenje Friedreichove ataksije
Spanish	Leriglitzona	Tratamiento de la ataxia de Friedreich
Swedish	Leriglitzon	Behandling av Friedreichs ataxi
Norwegian	Leriglitzon	Behandling av Friedreichs ataksi
Icelandic	Leriglitzon	Meðferð arfgengs mænuslingurs

¹ At the time of designation