



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Deferiprone for the treatment of neurodegeneration with brain iron accumulation

On 27 June 2018, orphan designation (EU/3/18/2034) was granted by the European Commission to Apotex Europe B.V., the Netherlands, for deferiprone for the treatment of neurodegeneration with brain iron accumulation.

What is neurodegeneration with brain iron accumulation?

Neurodegeneration with brain iron accumulation describes a group of inherited diseases characterised by a build-up of iron in the part of the brain involved in movement.

The diseases lead to brain damage and symptoms including progressive dystonia (worsening painful muscle spasms), spasticity (muscle stiffness), effects similar to Parkinson's disease such as shaking, muscle stiffness and slow movement, mental disorders including dementia, optic atrophy (damage to nerves in the eye) and retinal degeneration (damage to the back of the eye).

Neurodegeneration with brain iron accumulation is debilitating and life threatening because it results in reduced ability to walk, worsening vision and reduced life expectancy.

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

At the time of designation, neurodegeneration with brain iron accumulation affected approximately 0.03 in 10,000 people in the European Union (EU). This was equivalent to a total of around 2,000 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?

No satisfactory methods of treatment for neurodegeneration with brain iron accumulation were authorised in the EU at the time of orphan designation. Patients received supportive therapy including medicines for muscle problems and movement disorders to deal with the symptoms.

^{*}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?

Deferiprone is an iron chelator. This means that it attaches to iron in the body to form a compound that can be eliminated by the body, mainly in the urine. Deferiprone is expected to travel to the brain and reduce the build-up of iron in brain cells. This is expected to improve the symptoms in patients with neurodegeneration with brain iron accumulation.

What is the stage of development of this medicine?

The effects of deferiprone have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with deferiprone in patients with neurodegeneration with brain iron accumulation were ongoing.

At the time of submission, deferiprone was authorised throughout the EU for treatment of iron overload in patients with thalassaemia major.

At the time of submission, deferiprone was not authorised anywhere in the EU for neurodegeneration with brain iron accumulation. Orphan designation of the medicine has been granted in the United States for treatment of this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 May 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	Deferiprone	Treatment of neurodegeneration with brain iron accumulation
Bulgarian	Деферипрон	Лечение на невродегенерация с натрупване на желязо в мозъка
Croatian	Deferipron	Liječenje neurodegeneracije s nakupljanjem željeza u mozgu
Czech	Deferipron	Léčba neurodegenerace s akumulací železa v mozku
Danish	Deferipron	Behandling af neurodegeneration med jernophobning i hjernen
Dutch	Deferipron	Behandeling van neurodegeneratie met ijzerstapeling in de hersenen
Estonian	Deferipron	Ajus raua kuhjumisega kaasneva neurodegeneratsiooni ravi
Finnish	Deferiproni	Raudan aivoihin kertymiseen liittyvän neurodegeneraation hoito
French	Défériprone	Traitement de la neurodégénérescence avec accumulation de fer dans le cerveau
German	Deferipron	Behandlung von Neurodegeneration mit Eisenablagerung im Gehirn
Greek	Δεφεριπρόνη	Θεραπεία της νευροεκφύλισης με συσσώρευση σιδήρου στον εγκέφαλο
Hungarian	Deferipron	Agyi vasfelhalmozódással járó neurodegeneráció kezelése
Italian	Deferiprone	Cura della neurodegenerazione con accumulo cerebrale di ferro
Latvian	Deferiprons	Neirodēģenerācijas ar dzelzs uzkrāšanos smadzenēs ārstēšana
Lithuanian	Deferipronas	Geležies kaupimosi smegenyse sąlygotos neurodegeneracijos gydymas
Maltese	Deferipron	Trattamet għal newrodeġenerazzjoni b'akkumulazzjoni ta' ħadid fil-moħħ
Polish	Deferypron	Leczenie neurodegeneracji z odkładaniem żelaza w mózgu
Portuguese	Deferriprona	Tratamento da neurodegenerescência com acumulação cerebral de ferro
Romanian	Deferipronă	Tratamentul neurodegenerescenței cu acumulare cerebrală de fier
Slovak	Deferiprón	Liečba neurodegenerácie s akumuláciou železa v mozgu
Slovenian	Deferipron	Zdravljenje nevrodgeneracije z akumulacijo železa v možganih
Spanish	Deferiprona	Tratamiento de la neurodegeneración con acumulación de hierro en el cerebro
Swedish	Deferipron	Behandling av neurodegenerering med ackumulering av järn i hjärnan
Norwegian	Deferipron	Behandling av nevrodgenerasjon med jernavleiring i hjernen
Icelandic	Deferiprón	Meðferð við taugahrörnun með uppsöfnun járn í heila

¹ At the time of designation