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Public summary of opinion on orphan designation

9-cis, 12-cis-11,11-D2-linoleic acid ethyl ester for the treatment of infantile neuroaxonal dystrophy

On 26 February 2019, orphan designation (EU/3/19/2134) was granted by the European Commission to FGK Representative Service GmbH, Germany for 9-cis, 12-cis-11,11-D2-linoleic acid ethyl ester (also known as RT001) for the treatment of infantile neuroaxonal dystrophy.

What is infantile neuroaxonal dystrophy?

Infantile neuroaxonal dystrophy is a genetic disorder that mainly affects the nervous system. The condition usually starts in children before the age of 3 years, with the child's having difficulties with movement and slowly losing vision and intellectual skills.

The disease is caused by the build-up of substances in the nerves endings throughout the brain and body, which prevents them from working properly.

Infantile neuroaxonal dystrophy is a debilitating and life-threatening disease because of its progressive symptoms. The disease usually leads to death in childhood.

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

At the time of designation, infantile neuroaxonal dystrophy affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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 infantile neuroaxonal dystrophy. Patients received supportive treatment such as physiotherapy and medicines to relieve symptoms and improve the patient's general condition.

[*]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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

How is this medicine expected to work?

The medicine works by blocking production of the substances that build up in the nerves endings of patients with infantile neuroaxonal dystrophy. As a result, the medicine is expected to prevent the damages caused by their build-up in patients with the condition.

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, clinical trials with the medicine in patients with infantile neuroaxonal dystrophy were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for infantile neuroaxonal dystrophy. Orphan designation had been granted in the United States for PLA2G6–associated neurodegeneration.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 January 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 the 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	9-cis, 12-cis-11,11-D2-linoleic acid ethyl ester	Treatment of infantile neuroaxonal dystrophy
Bulgarian	Етилов естер на 9-цис, 12-цис-11,11-D2 линоленова киселина	Лечение на инфантилна невроаксонална дистрофия
Croatian	9-cis, 12-cis-11,11-D2- etilni ester linoleinske kiseline	Liječenje infantilne neuroaksonalne distrofije
Czech	9-cis, 12-cis-11,11-D2-ethylester kyseliny linolové	Léčba infantilní neuroaxonální dystrofie
Danish	9-cis, 12-cis-11,11-D2-linolensyreethylester	Behandling af infantil neuroaxonal dystrofi
Dutch	9-cis, 12-cis-11,11-D2-linoleïczuurethylester	Behandeling van infantiele neuroaxonale dysfrofie
Estonian	9-cis, 12-cis-11,11-D2-linoleenhappe etüülester	Infantiilse neuroaksonaalse düstroofia ravi
Finnish	9-cis, 12-cis-11,11-D2-linoleenihapon etyyliesteri	Infantiilin neuroaksonaalisen dystrofian hoito
French	9-cis, 12-cis-11,11-D2-acide linoléic éthyl ester	Traitement de la dystrophie neuroaxonale infantile
German	9-cis, 12-cis-11,11-D2-Linolsäure-ethyl-ester	Behandlung der infantilen neuroaxonalen Dystrophie
Greek	αιθυλικός εστέρας του 9-cis, 12-cis-11,11-D2 λινολεϊκού οξέως	Θεραπεία της βρεφικής νευροαξονικής δυστροφίας
Hungarian	9-cis, 12-cis-11, 11-D2-linolénsav etilészter	Infantilis neuroaxonalis dystrophia
Italian	9-cis, 12-cis-11,11-D2-etil ester dell'acido linoleico	Trattamento della distrofia neuroassonale infantile
Latvian	9-cis, 12-cis-11,11-D2-linolskābes etilesteris	Infantilas neiroaksonālās distrofijas ārstēšana
Lithuanian	9-cis, 12-cis-11,11-D2-linoleno rūgšties etilo esteris	Infantilinės neuroaksoninės distrofijos gydymas
Maltese	Ester tal-etil tal-aċidu 9-cis, 12-cis-11,11-D2-linolejku	Kura ta' distrofija newroassonali infantili
Polish	Ester kwasu 9-cis, 12-cis-11,11-D2-linolowego	Leczenie dziecięcej dystrofii neuroaksonalnej
Portuguese	Ester etílico do ácido 9-cis, 12-cis-11,11-D2-linoleico	Tratamento da distrofia neuroaxonal infantil
Romanian	Ester etilic al acidului 9-cis, 12-cis-11,11-D2-linoleic	Tratamentul distrofiei neuroaxonale infantile
Slovak	Etyl ester kyseliny 9-cis, 12-cis-11,11-D2 linolovej	Liečba infantilnej neuroaxonálnej dystrofie

[1] At the time of designation

Slovenian	9-cis, 12-cis-11,11-D2-linoleinska kislina, etilester	Zdravljenje infantilne nevroaksonske distrofije
Spanish	Etil ester del acido linoleic 9-cis, 12-cis-11,11-D2	Tratmiento de la distrofia neuroaxonal infantil
Swedish	9-cis, 12-cis-11,11-D2-linolsyra etylester	Behandling av infantil neuroaxonal dystrofi
Norwegian	9-cis, 12-cis-11,11-D2-linolsyreetylester	Behandling av infantil neuroaxonal dystrofi
Icelandic	9-cis,12-cis-11,11-D2-línólsýruetýlester	Meðferð á taugasímavisnun (neuroaxonal dystrophy) hjá ungbörnum