13 November 2015
EMA/COMP/603544/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide for the treatment of Duchenne muscular dystrophy

On 9 October 2015, orphan designation (EU/3/15/1560) was granted by the European Commission to FGK Representative Service GmbH, Germany, for N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide for the treatment of Duchenne muscular dystrophy.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy (DMD) is a genetic disease that gradually causes weakness and atrophy (wasting) of the muscles. It mainly affects boys, and is usually diagnosed before the age of six years. The muscle weakness usually starts in the hips and legs, before affecting the arms, chest and the heart. Patients with DMD lack normal dystrophin, a protein found in muscles. Because this protein helps to protect muscles from injury as muscles contract and relax, in patients with DMD the muscles become weaker and eventually stop working.

DMD causes long-term disability and is life threatening because of its effects on the heart and the respiratory muscles (muscles that are used to breathe). The disease usually leads to death in adolescence or early adulthood.

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

At the time of designation, DMD affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of 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).

*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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What treatments are available?

At the time of designation, Translarna (ataluren) was authorised in the EU to treat patients with DMD who have a specific type of mutation (change) called a nonsense mutation in their dystrophin gene. Patients also received supportive treatment such as physiotherapy.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with DMD; early studies in experimental models indicate that the medicine would not be limited to patients with a specific mutation and would therefore target a wider patient population compared with authorised treatments. 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?

It is thought that inflammation, particularly inflammation caused by the activity of a protein called 'NF-κB', leads to muscle damage and prevention of muscle regeneration seen in patients with DMD. This medicine is expected to work by reducing NF-κB activity. This is expected to reduce the muscle damage seen in DMD and enable muscle regeneration.

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 DMD had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for DMD. Orphan designation of the medicine has been granted in the United States for the treatment of DMD.

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide	Treatment of Duchenne muscular dystrophy
Bulgarian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-докоза-4,7,10,13,16,19-хексаенамидо)етил)-2-хидрокси бензамид	Лечение на мускулна дистрофия на Duchenne
Croatian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokoza-4,7,10,13,16,19-heksaenamido)etil)-2-hidroksi benzamid	Liječenje Duchenneove mišićne distrofije
Czech	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)-ethyl)-2-hydroxybenzamid	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)-ethyl)-2-hydroxybenzamid	Behandling af Duchenne muskeldystrofi
Dutch	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide	Behandeling van Duchenne spierdystrofie
Estonian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokosa-4,7,10,13,16,19-heksaenamido)etüül)-2-hüdroksü bensamiid	Duchenne'i lihasdüstroofia ravi
Finnish	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokosa-4,7,10,13,16,19-heksaaniamido)etyyli)-2-hydroksi bentsamidi	Duchennen lihasdystrofian hoito
French	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaénamido)éthyl)-2-hydroxy benzamide	Traitement de la dystrophie musculaire de Duchenne
German	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamid	Behandlung der Duchenne-Muskeldystrophie
Greek	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-δοκοσα-4,7,10,13,16,19-εξαενοαμιδο)εθυλ)-2-υδροξυ βενζαμίδιο	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokoza-4,7,10,13,16,19-hexaénamido)etil)-2-hidroxi benzamide	Duchenne dystrophia kezelése
Italian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-esaenammido)etil)-2-idrossi benzamide	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokosa-4,7,10,13,16,19-heksaēnamīd)etil)-2-hidroksi benzamīds	Dišēna muskuļu distrofijas ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokoza-4,7,10,13,16,19-heksaenamido)etil)-2-hidroksi benzamidas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokoza-4,7,10,13,16,19-heksaenamido)-etyl)-2-hydroksy benzamid	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)etil)-2-hidroxi benzamida	Tratamento da distrofia muscular de Duchenne
Romanian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)etil)-2-hidroxi benzamidă	Tratamentul distrofiei musculare Duchenne
Slovak	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokosa-4,7,10,13,16,19-hexaenamido)etyl)-2-hydroxy benzamid	Liečba Duchennovej muskulárnej dystrofie
Slovenian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokosa-4,7,10,13,16,19-heksaenamid)etil)-2-hidroksi benzamid	Zdravljenje Duchennove mišične distrofije
Spanish	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethil)-2-hidroxi benzamida	Tratamiento de la distrofia muscular de Duchenne
Swedish	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokosa-4,7,10,13,16,19-hexaenamido)etyl)-2-hydroxi bensamide	Behandling av Duchennes muskeldystrofi
Norwegian	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dokosa-4,7,10,13,16,19-heksaenamido) etyl)-2-hydroksy benzamid	Behandling av Duchennes muskeldystrofi
Icelandic	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-dókósa-4,7,10,13,16,19-hexaenamídó)ethýl)-2-hýdroxý benzamíð	Meðferð á Duchenne vöðvarýrnun