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EMA/696120/2017

Public summary of opinion on orphan designation

Tamoxifen citrate for the treatment of Duchenne muscular dystrophy

On 12 October 2017, orphan designation (EU/3/17/1944) was granted by the European Commission to Duchenne UK, United Kingdom, for tamoxifen citrate for the treatment of Duchenne muscular dystrophy.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy (DMD) is a genetic disease that causes increasing weakness and atrophy (wasting) of muscles. It mainly affects boys, and is usually diagnosed before the age of 6 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 damage 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 early adulthood.

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

At the time of designation, DMD 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, the medicine Translarna (ataluren) was authorised in the EU for the treatment of a small group of patients with DMD due to a particular type of mutation, called a nonsense mutation, in the dystrophin gene. Patients also received corticosteroids and supportive treatment such as physiotherapy.

*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 515,700,000 (Eurostat 2017).

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

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with DMD because laboratory data indicate that it is may be used to treat a wider range of DMD patients and not just those with a particular mutation. 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?

Tamoxifen citrate acts on oestrogen receptors (targets) on various cell types, including muscle cells, which has the effect of increasing strength and repairing muscles. Tamoxifen citrate is also expected to protect muscle cells from several types of damage that occurs in DMD.

What is the stage of development of this medicine?

The effects of tamoxifen citrate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with tamoxifen citrate in patients with DMD had been started.

At the time of submission, tamoxifen citrate was authorised in the EU for the treatment of breast cancer and infertility in women.

At the time of submission, tamoxifen citrate was not authorised anywhere in the EU for DMD or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 2017 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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Tamoxifen citrate	Treatment of Duchenne muscular dystrophy
Bulgarian	Тамоксифен цитрат	Лечение на мускулна дистрофия на Duchenne
Croatian	Tamoksifen citrat	Liječenje Duchenneove mišićne distrofije
Czech	Tamoxifencitrat	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Tamoxifen citrate	Behandling af Duchenne muskeldystrofi
Dutch	Tamoxifen citrate	Behandeling van Duchenne spierdystrofie
Estonian	Tamoksifeeni tsitraat	Duchenne'i lihasdüstroofia ravi
Finnish	Tamoksifeeni sitraatti	Duchennen lihasdystrofian hoito
French	Tamoxifène citrate	Traitement de la dystrophie musculaire de Duchenne
German	Tamoxifencitrat	Behandlung der Duchenne-Muskeldystrophie
Greek	Κιτρική ταμοξιφαίνη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Tamoxifen citrát	Duchenne dystrophia kezelése
Italian	Tamoxifene citrato	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Tamoksifēna citrāts	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Tamoksifenas citratas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Ċitrat tat-tamossifen	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Cytrynian tamoksyfenu	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Citrato de tamoxifeno	Tratamento da distrofia muscular de Duchenne
Romanian	Tamoxifen citrat	Tratamentul distrofiei musculare Duchenne
Slovak	Tamoxifen citrát	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Tamoksifen citrat	Zdravljenje Duchennove mišične distrofije
Spanish	Tamoksifeno citratas	Tratamiento de la distrofia muscular de Duchenne
Swedish	Tamoxifencitrat	Behandling av Duchennes muskeldystrofi
Norwegian	Tamoxifensitrat	Behandling av Duchennes muskeldystrofi
Icelandic	Tamoxifen sítrat	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation