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Public summary of opinion on orphan designation

Viltolarsen for the treatment of Duchenne muscular dystrophy

On 4 June 2020, orphan designation EU/3/20/2282 was granted by the European Commission to Medpace Finland Oy, Finland, for viltolarsen 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 muscles. It mainly affects boys, and usually starts 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 early adulthood.

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

At the time of designation, Duchenne muscular dystrophy 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 group of patients with DMD caused by a particular type of mutation (change), called a nonsense mutation, in the dystrophin gene. Patients also received supportive treatment such as physiotherapy.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with DMD because early studies indicate that the medicine may improve muscle function in patients with several other mutations in the dystrophin gene. 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?

Several mutations in the dystrophin gene prematurely stop the production of a normal dystrophin protein, leading to a shortened protein that does not function properly. This medicine is an antisense oligonucleotide (a small strand of synthetic genetic material). It is expected to work in patients with a deletion mutation (where a piece of the gene is missing) by enabling the protein-making apparatus in cells to move past the mutation, allowing the cells to produce a functional protein.

What is the stage of development of this medicine?

The effects of viltolarsen have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with viltolarsen in patients with DMD were ongoing.

At the time of submission, viltolarsen was not authorised anywhere in the EU for the treatment of DMD 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 23 April 2020, 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Viltolarsen	Treatment of Duchenne muscular dystrophy
Bulgarian	Вилтоларсен	Лечение на мускулна дистрофия на Duchenne
Croatian	Viltolarsen	Liječenje Duchenneove mišićne distrofije
Czech	Viltolarsen	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Viltolarsen	Behandling af Duchenne muskeldystrofi
Dutch	Viltolarsen	Behandeling van Duchenne spierdystrofie
Estonian	Viltolarseen	Duchenne'i lihasdüstroofia ravi
Finnish	Viltolarseeni	Duchennen lihasdystrofian hoito
French	Viltolarsen	Traitement de la dystrophie musculaire de Duchenne
German	Viltolarsen	Behandlung der Duchenne-Muskeldystrophie
Greek	Βιτολαρσένη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Viltolarsen	Duchenne dystrophia kezelése
Italian	Viltolarsen	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Viltolarsens	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Viltolarsenas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Viltolarsen	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Wiltolarsen	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Viltolarsen	Tratamento da distrofia muscular de Duchenne
Romanian	Viltolarsen	Tratamentul distrofiei musculare Duchenne
Slovak	Viltolarsen	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Viltolarsen	Zdravljenje Duchennove mišične distrofije
Spanish	Viltolarsen	Tratamiento de la distrofia muscular de Duchenne
Swedish	Viltolarsen	Behandling av Duchennes muskeldystrofi
Norwegian	Viltolarsen	Behandling av Duchennes muskeldystrofi
Icelandic	Viltolarsen	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation