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Public summary of opinion on orphan designation

Tetracosactide for the treatment of Duchenne muscular dystrophy

On 31 July 2018, orphan designation (EU/3/18/2054) was granted by the European Commission to Mallinckrodt Specialty Pharmaceuticals Ireland Limited, Ireland, for tetracosactide 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, DMD affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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 caused by a particular type of mutation (change), called a nonsense mutation, in the dystrophin gene. Patients also received 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 517,400,000 (Eurostat 2018).

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The sponsor has provided sufficient information to show that tetracosactide might be of significant benefit for patients with DMD. Data from laboratory studies show that the medicine reduces inflammation and this is associated with an improvement in measures of muscle function; in addition, the medicine can be used to treat all patients with the condition. 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?

Tetracosactide activates certain targets in cells called melanocortin receptors, which are thought to play an important role in reducing muscle damage and improving muscle function. This is expected to slow down the progression of the disease.

Tetracosactide is already authorised in the EU for treating inflammatory conditions.

What is the stage of development of this medicine?

The effects of tetracosactide 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 DMD were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 June 2018 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	Tetracosactide	Treatment of Duchenne muscular dystrophy
Bulgarian	Тетракосактид	Лечение на мускулна дистрофия на Duchenne
Croatian	Tetrakosaktid	Liječenje Duchenneove mišićne distrofije
Czech	Tetrakosaktid	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Tetracosactid	Behandling af Duchenne muskeldystrofi
Dutch	Tetracosactide	Behandeling van Duchenne spierdystrofie
Estonian	Tetrakosaktiid	Duchenne'i lihasdüstroofia ravi
Finnish	Tetrakosaktidi	Duchennen lihasdystrofian hoito
French	Tétracosactide	Traitement de la dystrophie musculaire de Duchenne
German	Tetrakosaktid	Behandlung der Duchenne-Muskeldystrophie
Greek	Τετρακοσακτίδη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Tetrakozaktid	Duchenne dystrophia kezelése
Italian	Tetracosactide	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Tetrakosaktīds	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Tetrakozaktidas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Tetrakosattur	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Tetrakosaktyd	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Tetracosactido	Tratamento da distrofia muscular de Duchenne
Romanian	Tetracosactidă	Tratamentul distrofiei musculare Duchenne
Slovak	Tetrakosaktid	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Tetrakozaktid	Zdravljenje Duchennove mišične distrofije
Spanish	Tetracosactida	Tratamiento de la distrofia muscular de Duchenne
Swedish	Tetrakosaktid	Behandling av Duchennes muskeldystrofi
Norwegian	Tetrakosaktid	Behandling av Duchennes muskeldystrofi
Icelandic	Tetrakósaktíð	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation