EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Asp-Arg-Val-Tyr-Ile-His-Pro for the treatment of Duchenne muscular dystrophy

On 19 February 2014, orphan designation (EU/3/14/1241) was granted by the European Commission to Gregory Fryer Associates Ltd, United Kingdom, for Asp-Arg-Val-Tyr-Ile-His-Pro 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 usually starts before the age of six years. The muscle weakness usually starts in the hips and legs, before reaching the chest, arms, and sometimes 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 damaged 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 around 25,000 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 511,100,000 (Eurostat 2014).

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What treatments are available?

At the time of designation, no satisfactory method had been authorised in the European Union to treat DMD. Treatment of patients with DMD primarily involved physiotherapy and other supportive treatments.

How is this medicine expected to work?

Because patients with DMD lack dystrophin, muscles get damaged with repetitive contractions, causing inflammation. This results in muscle fibrosis, where the damaged muscle cells develop scar tissue which contributes to the progressive muscle weakness in DMD patients.

The medicine is a form of a substance naturally found in the body, angiotensin (1-7). Its actions are not completely understood, but it is expected to counteract the effects of 'transforming growth factor beta' (TGF-beta), a protein which is involved in producing fibrosis, and thereby improve muscular function.

What is the stage of development of this medicine?

The effects of this 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 had been granted in the United States for treatment of skeletal muscle fibrosis and reduced muscle strength resulting from muscular dystrophy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 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:

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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	Asp-Arg-Val-Tyr-Ile-His-Pro	Treatment of Duchenne muscular dystrophy
Bulgarian	Asp (аспаргинова киселина) – Arg (аргинин) – Val (валин) – Tyr (тирозин) – Ile (изолевцин) - His (хистидин), Pro (пролин)	Лечение на мускулна дистрофия на Duchenne
Czech	Asp-Arg-Val-Tyr-Ile-His-Pro	Léčba pacientů s Duchennovou muskulární dystrofií
Croatian	Asp-Arg-Val-Tir-Ile-His-Pro	Liječenje Duchenneove mišićne distrofije
Danish	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling af Duchenne muskeldystrofi
Dutch	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandeling van Duchenne spierdystrofie
Estonian	Asp-Arg-Val-Tyr-Ile-His-Pro	Duchenne'i lihasdüstroofia ravi
Finnish	Asp-Arg-Val-Tyr-Ile-His-Pro	Duchennen lihasdystrofian hoito
French	Asp-Arg-Val-Tyr-Ile-His-Pro	Traitement de la dystrophie musculaire de Duchenne
German	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandlung der Duchenne-Muskeldystrophie
Greek	Asp-Arg-Val-Tyr-Ile-His-Pro	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Asp-Arg-Val-Tyr-Ile-His-Pro	Duchenne dystrophia kezelése
Italian	Asp-Arg-Val-Tyr-Ile-His-Pro	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Asp-Arg-Val-Tir-Ile-His-Pro	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Asp-Arg-Val-Tyr-Ile-His-Pro	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Asp-Arg-Val-Tyr-Ile-His-Pro	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Asp-Arg-Val-Tyr-Ile-His-Pro	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Asp-Arg-Val-Tyr-Ile-His-Pro	Tratamento da distrofia muscular de Duchenne
Romanian	Asp-Arg-Val-Tir-Ile-His-Pro	Tratamentul distrofiei musculare Duchenne
Slovak	Asp-Arg-Val-Tyr-Ile-His-Pro	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Asp-Arg-Val-Tir-Ile-His-Pro	Zdravljenje Duchennove mišične distrofije
Spanish	Asp-Arg-Val-Tyr-Ile-His-Pro	Tratamiento de la distrofia muscular de Duchenne
Swedish	Asp-Arg-Val-Tir-Ile-His-Pro	Behandling av Duchennes muskeldystrofi
Norwegian	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling av Duchennes muskeldystrofi
Icelandic	Asp-arg-val-týr-íle-his-pró	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation