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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised monoclonal antibody against myostatin for the treatment of Duchenne muscular dystrophy

On 8 February 2013, orphan designation (EU/3/13/1105) was granted by the European Commission to Pfizer Limited, United Kingdom, for humanised monoclonal antibody against myostatin 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 strengthen and protect muscles from injury as muscles contract and relax, in patients with DMD the muscles become weak 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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).

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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 of DMD. Treatment of patients with Duchenne muscular dystrophy primarily involved physiotherapy and other supportive treatments.

How is this medicine expected to work?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (an antigen) called myostatin. Myostatin is a protein present in muscle cells that prevents muscle growth and regeneration. When the medicine attaches to myostatin, it blocks its activity, which is expected to increase muscle mass and strength, thereby delaying the progression of the disease.

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 had been granted in the United States of America for DMD.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2013 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:

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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	Humanised monoclonal antibody against myostatin	Treatment of Duchenne muscular dystrophy
Bulgarian	Хуманизирано анти-миостатин моноклонално антитяло	Лечение на мускулна дистрофия тип Дюшен
Czech	Humanizovaná monoklonální protilátka proti myostatinu	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Humaniseret monoklonalt antistof mod myostatin	Behandling af Duchenne muskeldystrofi
Dutch	Gehumaniseerd anti-myostatine monoklonaal antilichaam	Behandeling van Duchenne spierdystrofie
Estonian	Inimese müostatiinivastane monoklonaalne antikeha	Duchenne'i lihasdüstroofia ravi
Finnish	Humanisoitu monoklonaalinen myostatiinin vasta-aine	Duchennen lihasdystrofian hoito
French	Anticorps monoclonal humanisé anti-myostatine	Traitement de la dystrophie musculaire de Duchenne
German	Humanisierter monoklonaler Antikörper gegen Myostatin	Behandlung der Duchenne-Muskeldystrophie
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι της μυοστατίνης	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Humanizált myostatin ellenes monoklonális antitest	Duchenne dystrophia kezelése
Italian	Anticorpo monoclonale umanizzato anti-miostatina	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Humanizēta monoklonālā miostatīna antiviela	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Žmogaus monokloninis antikūnas prieš miostatiną	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Antikorp monoklonali umanizzat kontra l-myostatin	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Humanizowane przeciwciało monoklonalne przeciw miostatynie	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Anticorpo monoclonal humanizado anti-miostatina	Tratamento da distrofia muscular de Duchenne
Romanian	Anticorp monoclonal umanizat anti-miostatina	Tratamentul distrofiei musculare Duchenne

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Humanizovaná monoklonálna protilátka proti myostatínu	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Humanizirano monoklonsko protitelo proti miostatinu	Zdravljenje Duchennove mišične distrofije
Spanish	Anticuerpo monoclonal humanizado anti-miostatina	Tratamiento de la distrofia muscular de Duchenne
Swedish	Humaniserad monoklonal antikropp mot myostatin	Behandling av Duchennes muskeldystrofi
Norwegian	Humanisert anti-myostatin monoklonalt antistoff	Behandling av Duchennes muskeldystrofi
Icelandic	Mannaaðlagað einstofna mótefni gegn mýóstatíni	Meðferð á Duchenne vöðvarýrnun