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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride for the treatment of Duchenne muscular dystrophy

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Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 26 April 2013, orphan designation (EU/3/13/1122) was granted by the European Commission to N-GENE Kutási és Fejlesztesi Kf, Hungary, for R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride 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.

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

At the time of designation, DMD affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of approximately 15,000 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, 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?

The medicine is a small substance that has been designed to increase the production of a protein called heat shock protein 72 (Hsp 72). Heat shock proteins are proteins whose production is increased when the cell is exposed to factors such as stress and inflammation. Hsp 72 protects muscle cells by blocking the production of molecules called cytokines involved in the process of inflammation. In addition, Hsp72 is expected to improve the functioning of a pump in the muscle cells that is responsible for moving calcium into the cells and whose activity is known to be decreased in DMD. By increasing the production of Hsp 72, the medicine is expected to improve muscle strength, thereby slowing down the progression of the disease.

What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicinal product in patients with condition had been started.

At the time of submission, the medicinal product 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 13 March 2013 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.

*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.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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	R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride	Treatment of Duchenne muscular dystrophy
Bulgarian	R, S-O-(3- пиперидино-2-хидрокси-1-пропил)- никотинова киселина амидохим дихидро хлорид	Лечение на мускулна дистрофия на Duchenne
Czech	R, S-O-(3-piperidinovou-2-hydroxy-1-propyl) - kyselina nikotinová amidoxime dihydrochlorid	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	R, S-O-(3-piperidino-2-hydroxy-1-propyl) - nicotinsyre amidoxim dihydrochlorid	Behandling af Duchenne muskeldystrofi
Dutch	R, S-O-(3-piperidino-2-hydroxy-1-propyl) - nicotinezuur amidoxime dihydrochloride	Behandeling van Duchenne spierdystrofie
Estonian	R, S-O-(3-piperidino-2-hüdroksü-1-propüül) - nikotiinhappe amidoxime dihüdrokloriid	Duchenne'i lihasdüstroofia ravi
Finnish	R, S-O-(3-piperidino-2-hydroksi-1-propyyli)- nikotiinihappo amidoksiimi dihydrokloridi	Duchennen lihasdystrofian hoito
French	R, S-O-(3-pipéridino-2-hydroxy-1-propyl) - nicotinique dichlorhydrate amidoxime d'acide	Traitement de la dystrophie musculaire de Duchenne
German	R,S-O-(3-Piperidino-2-hydroxy-1-propyl) - Nikotinsäure Amidoxim Dihydrochlorid	Behandlung der Duchenne-Muskeldystrophie
Greek	Διυδροχλωρική R, S-O-(3-πιπεριδινο-2-υδροξυ-1-προπυλο) - αμιδοξίμη νικοτινικού οξέος-	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	R, S-O-(3-piperidino-2-hidroxi-1-propil) - nikotinsav amidoxim dihidroklorid	Duchenne dystrophia kezelése
Italian	R, S-O-(3-piperidino-2-idrossi-1-propil) - acido nicotinico dicloridrato amidoxime	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	R, S-O-(3-piperidino-2-hidroksi-1-propil) - nikotīnskābes amidoksīma dihidrohlorīds	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	R, S-O-(3-piperidino-2-hidroksi-1-propil) - nikotino rūgšties amidoksimo dihidrochloridas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	R, S-O-(3-piperidino-2-hydroxy-1-propyl) - nicotinic acid amidoxime dihydrochloride	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Dwuchlorowodorek amidoksymu R, S-O-(3-piperydyno-2-hydroksy-1-propylo) - kwasu nikotynowego	Leczenie zaniku mięśni typu Duchenne'a

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	R, S-O-(3-piperidino-2-hidroxi-1-propil) - dicloridrato de amidoxima de ácido nicotínico	Tratamento da distrofia muscular de Duchenne
Romanian	R, S-O-(3-piperidino-2-hidroxi-1-propil) acid nicotinic amidoximă diclorhidrat	Tratamentul distrofiei musculare Duchenne
Slovak	R, S-O-(3-piperidín-2-hydroxy-1-propyl) - kyselina nikotínová amidoxim dihydrochlorid	Liečba Duchennovej muskulárnej dystrofie
Slovenian	R, S-O-(3-piperidino-2-hidroksi-1-propil) - amidoksim dihidroklorid nikotinsko kislino	Zdravljenje Duchennove mišične distrofije
Spanish	R, S-O-(3-piperidino-2-hidroxi-1-propil) - dihidrocloruro de ácido nicotínico amidoxima	Tratamiento de la distrofia muscular de Duchenne
Swedish	R, S-O-(3-piperidino-2-hydroxy-1-propyl) - nikotinsyra amidoxim-dihydroklorid	Behandling av Duchennes muskeldystrofi
Norwegian	R, S-O-(3-piperidino-2-hydroksy-1-propyl)-nikotinsyreamidoksimdihydroklorid	Behandling av Duchennes muskeldystrofi
Icelandic	R, S-O-(3-píperidínó-2-hýdroxý-1-própýlparabensóat) - nikótínsýru amidoxím díhýdróklóríð	Meðferð á Duchenne vöðvarýrnun