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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione for the treatment of Duchenne muscular dystrophy

First publication	23 September 2014
Rev.1: transfer of sponsorship	21 May 2015

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 22 August 2014, orphan designation (EU/3/14/1309) was granted by the European Commission to NDA Group AB, Sweden, for 17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione for the treatment of Duchenne muscular dystrophy.

The sponsorship was transferred to ReveraGen BioPharma Limited, United Kingdom, in May 2015.

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 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 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 less than 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 41,000 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 EU 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 expected to reduce the inflammation that occurs in the muscles of patients with DMD. It works by blocking the production of certain inflammatory substances called cytokines. This is expected to reduce the inflammation, strengthen the affected muscles and improve the symptoms of patients with DMD.

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 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 this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 July 2014 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.

*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 512,900,000 (Eurostat 2014).

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	17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione	Treatment of Duchenne muscular dystrophy
Bulgarian	17α,21-дихидрокси-16α-метил-прегна-1,4,9(11)-триен-3,20-дион	Лечение на мускулна дистрофия на Duchenne
Croatian	17α,21-dihidroksi-16α-metil-pregna-1,4,9(11)-trien-3,20-dion	Liječenje Duchenneove mišićne distrofije
Czech	17α,21-dihydroxy-16α-metyl-pregna-1,4,9(11)-trien-3,20-dion	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-trien-3,20-dion	Behandling af Duchenne muskeldystrofi
Dutch	17-alfa,21-dihydroxy-16-alfa-methylpregna-1,4,9(11)-trieen-3,20-dion	Behandeling van Duchenne spierdystrofie
Estonian	17α,21-dihüdroksü-16α-metüül-pregna-1,4,9(11)-trieen-3,20-dioon	Duchenne'i lihasdüstroofia ravi
Finnish	17α,21-dihydroksi-16α-metyylipregna-1,4,9(11)-trieeni-3,20-dioni	Duchennen lihasdystrofian hoito
French	17α,21-dihydroxy-16α-méthylprégna-1,4,9(11)-trièn-3,20-dione	Traitement de la dystrophie musculaire de Duchenne
German	17α,21-Dihydroxy-16α-methyl-pregna-1,4,9(11)-trien-3,20-dion	Behandlung der Duchenne-Muskeldystrophie
Greek	17α,21-διυδροξυ-16α-μεθυλοπρεγνα-1,4,9(11)-τριενο-3,20-διόνη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	17α,21-dihidroxi-16α-metil-pregna-1,4,9(11)-trién-3,20-dion	Duchenne dystrophia kezelése
Italian	17α,21-diidrossi-16α-metil-pregna-1,4,9(11)-triene-3,20-dione	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	17α,21-dihidroksi-16α-metil-pregna-1,4,9(11)-triēn-3,20-dions	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	17α,21-dihidroksi-16α-metil-pregna-1,4,9(11)-trien-3,20-dionas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	17α,21-dihydroksy-16α-metylo-pregna-1,4,9(11)-trieno-3,20-dion	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	17α,21-diidroxi-16α-metil-pregna-1,4,9(11)-trieno-3,20-diona	Tratamento da distrofia muscular de Duchenne
Romanian	17α,21-dihidroxi-16α-metil-pregna-1,4,9(11)-trien-3,20-dionă	Tratamentul distrofiei musculare Duchenne
Slovak	17α,21-dihydroxy-16α-metylpregna-1,4,9(11)-trién-3,20-dión	Liečba Duchennovej muskulárnej dystrofie

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	17α,21-dihidroksi-16α-metil-pregna-1,4,9(11)-trien-3,20-dion	Zdravljenje Duchennove mišične distrofije
Spanish	17α,21-dihidroxi-16α-metil-pregna-1,4,9(11)-trieno-3,20-diona	Tratamiento de la distrofia muscular de Duchenne
Swedish	17α,21-dihydroxi-16α-metylpregna-1,4,9(11)-trien-3,20-dion	Behandling av Duchennes muskeldystrofi
Norwegian	17α,21-dihydroksy-16α-metyl-pregna-1,4,9(11)-trien-3,20-dion	Behandling av Duchennes muskeldystrofi
Icelandic	17α,21-díhýdroxý-16α-metýl-pregna-1,4,9(11)-tríen-3,20-díón	Meðferð á Duchenne vöðvarýrnun