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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Halofuginone hydrobromide 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 2012, orphan designation (EU/3/12/988) was granted by the European Commission to Biological Consulting Europe Ltd, United Kingdom, for halofuginone hydrobromide 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 symptoms usually start 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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

At the time of designation, DMD affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 20,000 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 submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for treatment of the condition. Treatment of patients with Duchenne muscular dystrophy primarily involves 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 accumulate too much collagen-rich, fibrous tissue that contributes to the progressive muscle weakness in DMD patients.

Halofuginone hydrobromide is expected to reduce muscle fibrosis by blocking the effects of 'transforming growth factor beta' (TGF-beta), a protein found in high levels in muscle and plasma of DMD patients, which is involved in the production of excess collagen. By reducing the production of excess collagen, this medicine is expected to reduce the fibrosis and improve muscular function.

In addition, halofuginone hydrobromide is also expected to have anti-inflammatory effects and to increase muscle regeneration.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of halofuginone hydrobromide in experimental models was ongoing.

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 treatment of DMD.

Orphan designation has been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 March 2012 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;

*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.
At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

- 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:

Biological Consulting Europe Ltd
Wallace Building
Roslin BioCentre
Roslin
Midlothian EH25 9PP
United Kingdom
Tel. +44 (0)131 440 6480
Fax +44 (0)131 440 6486
E-mail: info@bioconsulteurope.com

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	Halofuginone Hydrobromide	Treatment of Duchenne muscular dystrophy
Bulgarian	халофугинон хидробромид	Лечение на мускулна дистрофия на Duchenne
Czech	Halofuginon hydrobromid	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Halofuginon hydrobromid	Behandling af Duchenne muskeldystrofi
Dutch	Halofuginone hydrobromide	Behandeling van Duchenne spierdystrofie
Estonian	Halofuginoonhüdrobromiid	Duchenne'i lihasdüstroofia ravi
Finnish	Halofuginonihydrobromidi	Duchennen lihasdystrofian hoito
French	Halofuginone hydrobromide	Traitement de la dystrophie musculaire de Duchenne
German	Halofuginon hydrobromid	Behandlung der Duchenne-Muskeldystrophie
Greek	Αλοφουγινόνη Υδροβρωμίδη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Halofuginon hidrobromid	Duchenne dystrophia kezelése
Italian	Alofuginone idrobromuro	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Halofuginona hidrobromīds	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Halofuginono hidrobromidas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Halofuginone hydrobromide	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Bromowodorek halofuginonu	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Bromidrato de Halofuginona	Tratamento da distrofia muscular de Duchenne
Romanian	Bromhidrat de halofuginonă	Tratamentul distrofiei musculare Duchenne
Slovak	Halofuginón hydrobromid	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Halofuginon hidrobromid	Zdravljenje Duchennove mišične distrofije
Spanish	Hidrobromuro de halofuginona	Tratamiento de la distrofia muscular de Duchenne
Swedish	Halofuginon hydrobromid	Behandling av Duchennes muskeldystrofi
Norwegian	Halofuginon hydrobromid	Behandling av Duchennes muskeldystrofi
Icelandic	Halófúgínón hýdróbrómíð	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation