19 July 2012
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Givinostat for the treatment of Duchenne muscular dystrophy

On 4 July 2012, orphan designation (EU/3/12/1009) was granted by the European Commission to Italfarmaco S.p.A., Italy, for givinostat 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 is 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).

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 involved physiotherapy and other supportive treatments.

[*]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 506,300,000 (Eurostat 2011).

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How is this medicine expected to work?

Givinostat is an 'HDAC inhibitor' medicine. This means that it blocks enzymes called histone deacetylases (HDACs), which are involved in turning genes 'on' and 'off' within cells. By blocking HDAC enzymes, givinostat is expected to 'switch on' the follistatin gene, thereby increasing the amount of the follistatin protein in muscle cells. Follistatin is expected to increase muscle mass and prevent muscle degeneration by opposing the effects of myostatin, a protein that causes fat and fibrotic tissue to build up in the muscle preventing muscle growth and regeneration. This is expected to improve the symptoms of DMD.

What is the stage of development of this medicine?

The effects of givinostat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with givinostat in patients with DMD were ongoing.

At the time of submission, givinostat 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 11 May 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;
- 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	Givinostat	Treatment of Duchenne muscular dystrophy
Bulgarian	Гивиностат	Лечение на мускулна дистрофия тип Дюшен
Czech	Givinostat	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Givinostat	Behandling af Duchenne muskeldystrofi
Dutch	Givinostat	Behandeling van Duchenne spierdystrofie
Estonian	Givinostat	Duchenne'i lihasdüstroofia ravi
Finnish	Givinostaatti	Duchennen lihasdystrofian hoito
French	Givinostat	Traitement de la dystrophie musculaire de Duchenne
German	Givinostat	Behandlung der Duchenne-Muskeldystrophie
Greek	Τζιβινοστάτη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Givinostat	Duchenne dystrophia kezelése
Italian	Givinostat	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Givinostats	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Givinostatas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Givinostat	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Giwinostat	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Givinostate	Tratamento da distrofia muscular de Duchenne
Romanian	Givinostat	Tratamentul distrofiei musculare Duchenne
Slovak	Givinostat	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Givinostat	Zdravljenje Duchennove mišične distrofije
Spanish	Givinostat	Tratamiento de la distrofia muscular de Duchenne
Swedish	Givinostat	Behandling av Duchennes muskeldystrofi
Norwegian	Givinostat	Behandling av Duchennes muskeldystrofi
Icelandic	Gívónóstat	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation