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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Idebenone for the treatment of Duchenne muscular dystrophy

On 20 March 2007, orphan designation (EU/3/07/437) was granted by the European Commission to Santhera Pharmaceuticals (Deutschland) GmbH, Germany, for idebenone for the treatment of Duchenne muscular dystrophy.

The name of the sponsor changed to Santhera Pharmaceuticals (Deutschland) GmbH in September 2010.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy is an inherited genetic disease, which usually starts before the age of 6. It is characterised by progressive weakness of the muscles, first involving the hips and legs, and later also the muscles of the chest and arms. Genes located on structures present in each cell of the body (the chromosomes) carry the information that characterises each individual. In humans, the so-called X and Y-chromosomes determine the sex (males have one X and one Y, females have 2 Xs), but carry also other genetic information. Duchenne muscular dystrophy is caused by an abnormality of a gene located on the X chromosome. This gene is responsible for the production of a protein, dystrophin, in the muscle cells. This means that patients suffering from this condition do not produce the dystrophin protein, or produce a non-functional dystrophin. As boys, contrary to girls, only have one X chromosome, and thus one single copy of dystrophin gene, they have a much higher probability of suffering from Duchenne muscular dystrophy. Duchenne muscular dystrophy is chronically debilitating and life-threatening.

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

At the time of designation, Duchenne muscular dystrophy affected approximately 0.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 23,000 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 based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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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?

Mitochondria are structures located inside the cells, which produce the energy necessary for the cell functioning, through a process named "cellular respiration" which requires oxygen to produce energy. During cellular respiration, some toxic forms of oxygen (called oxygen free radicals) can be produced; these must be neutralised by other substances to avoid cell damage. Idebenone is expected to act as a neutraliser for these toxic forms of oxygen. Thus, idebenone is expected to have an antioxidant effect, and consequently prevent cellular damage.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the effects of idebenone had been evaluated in experimental models, and clinical trials in patients with Duchenne muscular dystrophy were ongoing.

Idebenone was not authorised anywhere in the world for treatment of Duchenne muscular dystrophy or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 February 2007 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	Idebenone	Treatment of Duchenne muscular dystrophy
Bulgarian	Идебенон	Лечение на мускулна дистрофия на Duchenne
Czech	Idebenon	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	Idebenon	Behandling af Duchenne muskeldystrofi
Dutch	Idebenone	Behandeling van Duchenne spierdystrofie
Estonian	Idebenoon	Duchenne'i lihasdüstroofia ravi
Finnish	Idebenoni	Duchennen lihasdystrofian hoito
French	Idébénone	Traitement de la dystrophie musculaire de Duchenne
German	Idebenon	Behandlung der Duchenne-Muskeldystrophie
Greek	Ιδεβενόνη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Idebenone	Duchenne dystrophia kezelése
Italian	Idebenone	Trattamento di distrofia muscolare di tipo Duchenne
Latvian	Idebenons	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Idebenonas	Duchenne (Diušeno) raumenų distrofijos gydymas
Polish	Idebenon	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Idebenona	Tratamento da distrofia muscular de Duchenne
Romanian	Idebenonă	Tratamentul distrofiei musculare Duchenne
Slovak	Idebenon	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Idebenon	Zdravljenje Duchennove mišične distrofije
Spanish	Idebenona	Tratamiento de la distrofia muscular de Duchenne
Swedish	Idebenon	Behandling av Duchennes muskeldystrofi
Norwegian	Idebenon	Behandling av Duchennes muskeldystrofi
Icelandic	Ídebenón	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation