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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Idebenone for the treatment of Friedreich's ataxia

On 8 March 2004, orphan designation (EU/3/04/189) was granted by the European Commission to Promedipharm GmbH, Germany, for idebenone for the treatment of Friedreich's ataxia.

The sponsorship was transferred to Santhera Pharmaceuticals (Deutschland) AG in December 2005.

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

What is Friedreich's ataxia?

Friedreich's ataxia is an inherited (genetic) disease of the nervous system and muscles. Genes located on structures called chromosomes carry the genetic information that determines the characteristics of each individual. In humans, each cell has 23 pairs of chromosomes. For each pair one chromosome is inherited from the mother and the other from the father. In Friedreich's ataxia there is an abnormality in a gene carried by the pair number 9 and responsible for the production of a protein called "frataxin". For a patient to be affected of Friedreich's ataxia both chromosomes on the pair number 9 should be affected. Frataxin plays a major role in iron metabolism and its absence would produce the accumulation of iron and other substances that finally would injure the cells, in particula those of the nervous system.

Friedreich's ataxia is characterised by difficulties to coordinate movements. The symptoms develop progressively and are mainly due to the neurological alterations. They mainly concern the ability to walk, gait disturbances, speech problems, cardiac function (cardiomyopathy) and are sometimes associated to diabetes.

Friedreich's ataxia is chronically debilitating and life threatening due to the progression of symptoms, severe neurological and cardiac complications and short life expectancy.

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

At the time of designation, Friedreich's ataxia affected approximately 0.7 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 27,000 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?

No satisfactory methods exist that were authorised at the time of application.

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". This process is realised by a complex of different molecules that need oxygen to produce energy. During this process, toxic oxygen free radicals can be produced and they should be neutralised by other substances in order not to be harmful for the cell. One of the hypotheses for Friedreich's ataxia is that due to the frataxin protein deficit, the iron overload in the mitochondria, could lead to the formation of an excess of toxic oxygen free radicals. Idebenone is expected to act as a reactive oxygen scavenger. That means that it can naturally accept and link oxygen molecules and therefore have an antioxidant effect. By binding these reactive oxygen molecules idebenone can thereby prevent cellular damage.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with Friedreich's ataxia were ongoing.

Idebenone was not marketed anywhere worldwide for Friedreich's ataxia 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 5 February 2004 recommending the granting of this designation.

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[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition.

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 Friedreich's ataxia
Czech	Idebenon	Léčba Friedrichovy ataxie
Danish	Idebenon	Behandling af Friedreichs ataksi
Dutch	Idebenone	Behandeling van de ataxie van Friedreich
Estonian	Idebenoon	Friedreichi ataksia ravi
Finnish	Idebenoni	Friedreich-ataksian hoito
French	Idébénone	Traitement de l'ataxie de Friedreich
German	Idebenon	Therapie der Friedreichschen Ataxie
Greek	Ιδεβενόνη	Θεραπεία της ataξίας Friedreich
Hungarian	Idebenone	Friedreich ataxia kezelése
Italian	Idebenone	Trattamento dell'atassia di Friedreich
Latvian	Idebenons	Frīdreiha ataksijas ārstēšana
Lithuanian	Idebenonas	Fridreicho ataksijos gydymas
Polish	Idebenon	Leczenie ataksji Friedricha
Portuguese	Idebenona	Tratamento da ataxia de Friedreich
Slovak	Idebenon	Liečba Friedreichovej ataxie
Slovenian	Idebenon	Zdravljenje Friedreichove atakcije
Spanish	Idebenona	Tratamiento de la ataxia de Friedreich
Swedish	Idebenon	Behandling av Friedreichs ataxi

[1] At the time of transfer of sponsorship