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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Idebenone for the treatment of Leber's hereditary optic neuropathy

On 15 February 2007, orphan designation (EU/3/07/434) was granted by the European Commission to Santhera Pharmaceuticals (Deutschland) GmbH, Germany, for idebenone for the treatment of Leber's hereditary optic neuropathy.

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

What is Leber's hereditary optic neuropathy?

Leber's hereditary optic neuropathy is an inherited disease characterised by progressive loss of sight. Patients affected by Leber's hereditary optic neuropathy have mutations in the genetic material of mitochondria. Mitochondria are structures located inside cells, which produce the energy necessary for cells to function. It is thought that the mutations lead to a defective function of the mitochondria in the optic nerve cells and to degeneration of these cells that are needed for vision. Leber's hereditary optic neuropathy is chronically debilitating due to progressive loss of vision.

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

At the time of designation, Leber's hereditary optic neuropathy affected less than 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 46,000 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. Patients with Leber's hereditary optic neuropathy usually receive genetic counselling and general support such as information and regular medical follow up.

[*]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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How is this medicine expected to work?

Mitochondria produce the energy necessary for the cell functioning through a process named "cellular respiration" which requires oxygen and produces 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 cellular damage. Idebenone is expected to act as a neutraliser of 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 Leber's hereditary optic neuropathy were planned.

Idebenone was not authorised anywhere in the world for treatment of Leber's hereditary optic neuropathy 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 10 January 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 Leber's hereditary optic neuropathy
Bulgarian	Идебенон	Лечение на наследствена оптична невропатия на Leber
Czech	Idebenon	Léčba Leberovy hereditární optické neuropatie
Danish	Idebenon	Behandling af Lebers hereditære opticusneuropati
Dutch	Idebenone	Behandeling van Leber hereditaire optische neuropathie
Estonian	Idebenoon	Leberi päriliku optilise neuropaatia ravi
Finnish	Idebenoni	Leberin atrofian hoito
French	Idébénone	Traitement de la neuropathie optique héréditaire de Leber
German	Idebenon	Behandlung der Leberschen hereditären Optikusneuropathie
Greek	Ιδεβενόνη	Θεραπεία κληρονομικής οπτικής νευροπάθειας του Leber
Hungarian	Idebenone	Leber-féle hereditaer opticus neuropathia kezelése
Italian	Idebenone	Trattamento della neuropatia ottica ereditaria di Leber
Latvian	Idebenons	Lēbera hereditārās optiskās neiropātijas ārstēšana
Lithuanian	Idebenonas	Lėberio paveldimosios optinės neuropatijos gydymas
Polish	Idebenon	Leczenie dziedzicznej neuropatii Lebera nerwu wzrokowego
Portuguese	Idebenona	Tratamento de neuropatia óptica hereditária de Leber
Romanian	Idebenonă	Tratamentul neuropatiei optice ereditare Leber
Slovak	Idebenon	Liečba Leberovej dedičnej neuropatie optického nervu
Slovenian	Idebenon	Zdravljenje Leberjeve hereditarne optične nevropatije
Spanish	Idebenona	Tratamiento de la neuropatía óptica hereditaria de Leber
Swedish	Idebenon	Behandling av Lebers hereditära optikusneuropati
Norwegian	Idebenon	Behandling av Lebers hereditære optikusneuropati
Icelandic	Ídebenón	Meðferð við arfgengum Lebers sjóntaugarkvilla

[1] At the time of designation