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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Alpha-tocotrienol quinone for the treatment of Leigh syndrome

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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 9 December 2011, orphan designation (EU/3/11/937) was granted by the European Commission to Edison Europe Holding BV, the Netherlands, for alpha-tocotrienol quinone for the treatment of Leigh syndrome.

What is Leigh syndrome?

Leigh syndrome, also known as subacute necrotising encephalomyelopathy, is an inherited disease of the brain and the nervous system leading to problems such as poor motor function (movement), weakness, lack of muscle tone and difficulty breathing. Symptoms typically start before the age of 12 months.

The disease has many genetic causes, all of which are linked to defects in the mitochondria, the energy-producing components within cells.

Leigh syndrome is debilitating and life threatening due to progressive worsening of nervous system function as well as problems with heart, kidney and hormonal glands.

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

At the time of designation, Leigh syndrome affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 people*, and is below the ceiling

*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 507,700,000 (Eurostat 2011).



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 of treatment were authorised in the EU at the time of designation. Patients received supportive therapy to deal with the symptoms such as mechanical support with breathing, vitamins and supplements.

How is this medicine expected to work?

Alpha-tocotrienol quinone is manufactured from vitamin E and is given by mouth. It is expected to enter cells of the central nervous system, where it will increase the levels of a substance called glutathione. Low levels of glutathione are seen in patients with Leigh syndrome and are thought to be linked to the development of the symptoms of the disease.

What is the stage of development of this medicine?

The effects of alpha-tocotrienol quinone have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with alpha-tocotrienol quinone in patients with Leigh syndrome were ongoing.

At the time of submission, alpha-tocotrienol quinone was not authorised anywhere in the EU for Leigh syndrome. Orphan designation of alpha-tocotrienol quinone had been granted in the United States of America for the treatment of inherited mitochondrial respiratory chain diseases.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2011 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:

Edison Orphan Pharma BV
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11th floor
3029 AK Rotterdam
The Netherlands
Tel. +1 650 641 9206 / +31 6 20 44 30 98
<http://edisonpharma.com/contact/>

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Alpha-tocotrienol quinone	Treatment of Leigh syndrome
Bulgarian	Алфа-токоτριенол хинон	Лечение на синдрома на Leigh
Czech	Alfa-tokotrienol chinon	Léčba syndromu Leigh
Danish	Alpha-tocotrienol quinon	Behandling af Leighs syndrom
Dutch	Alpha-tocotriënol chinon	Behandeling van Leigh syndroom
Estonian	Alfa-tocotrienool kinoon	Leigh sündroomi ravi
Finnish	Alfa-tokotrienoli kinoni	Leigh:n oireyhtymän hoito
French	Alpha-tocotriénol quinone	Traitement de syndrome de Leigh
German	Alpha-Tocotrienol Chinon	Behandlung des Leigh-Syndroms
Greek	Αλφα-τοκοτριενόλη κινόνης	Θεραπεία του συνδρόμου Leigh
Hungarian	Alfa-tocotrienol kinon	Leigh-szindróma kezelése
Italian	Alfa-tocotrienolo chinone	Trattamento della sindrome di Leigh
Latvian	Alfa-tokotrienol ginīns	Leiga (Leigh) sindroma ārstēšana
Lithuanian	Alfa-tokotrienol kvinonas	Leigh sindromo gydymas
Maltese	Alfa-kinone tocotrienol	Kura tas-sindrome ta' Leigh
Polish	Chinon alfa-tokotrienolu	Leczenie zespołu Leigha
Portuguese	Alfa-tocotrienol quinona	Tratamento da síndrome de Leigh
Romanian	Alfa-tocotrienol chinonă	Tratamentul sindromului Leigh
Slovak	Alfa-tokotrienolov chinon	Liečba Leighovho syndrómu
Slovenian	Alfa-tokotrienol kinon	Zdravljenje sindroma Leigh
Spanish	Alfa-tocotrienol quinona	Tratamiento del síndrome de Leigh
Swedish	Alfa-tokotrienolkinon	Behandling av Leighs syndrom
Norwegian	Alfatokotrienolkinon	Behandling av Leighs syndrom
Icelandic	Alfa tócotríenól quínón	Meðferð á Leigh heilkenni

¹ At the time of designation