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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Omigapil maleate for the congenital muscular dystrophy with merosin (laminin alpha 2) deficiency

On 8 May 2008, orphan designation (EU/1/08/544) was granted by the European Commission to Santhera Pharmaceuticals (Deutschland) GmbH, Germany, for omigapil maleate for the treatment of congenital muscular dystrophy with merosin (laminin alpha 2) deficiency.

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

What is congenital muscular dystrophy with merosin (laminin alpha 2) deficiency?

Congenital muscular dystrophies (CMD) are a group of hereditary disorders, frequently presenting at birth or within the first six months of life. There are many different forms of CMDs and each form is caused by a specific defect in a gene. All forms of CMD share some symptoms and signs; such as weakness and degeneration of muscles, contractures and joint deformities. Usually CMD leads to difficulty in movement, skeletal deformation (scoliosis) and respiratory failure. Mental retardation is sometimes present. The most common form of CMD is caused by a deficiency of a protein called laminin alpha 2 (also called merosin). Laminins are found in tissues where they provide support to the cells and they also have other functions such as protecting the cells from dying. Laminin alpha 2 supports muscle cells and a deficiency of laminin alpha 2 in muscle tissue leads to increased muscle cell death and progressive muscle weakness. Congenital muscular dystrophy with merosin (laminin alpha 2) deficiency is a chronically debilitating and life-threatening disease.

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

At the time of designation, congenital muscular dystrophy with merosin (laminin alpha 2) deficiency affected approximately 0.04 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 2,000 people, and is below the ceiling for orphan designation, which is 5 people in 10,000.

*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 27), Norway, Iceland and Lichtenstein. This represents a population of 502,282,000 (Eurostat 2008). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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?

The exact mechanism of action of omigapil maleate is not known. However, it is thought that the product interacts with a protein called glyceraldehyde 3-phosphate dehydrogenase, which is involved in cell death. By interacting with this protein, omigapil maleate may protect muscle cells from dying.

What is the stage of development of this medicine?

The effects of omigapil maleate were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with congenital muscular dystrophy with merosin (laminin alpha 2) deficiency were initiated.

Omigapil maleate was not authorised anywhere worldwide for congenital muscular dystrophy with merosin (laminin alpha 2) deficiency 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 4 March 2008 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:

Santhera Pharmaceuticals (Deutschland) GmbH
Wallbrunnstrasse 24
D-79539 Lörrach
Germany
Telephone: +49 7621 1690 200
Telefax: +49 7621 1690 201
E-mail: klaus.schollmeier@santhera.com

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	Omigapil maleate	Treatment of congenital muscular dystrophy with merosin (laminin alpha 2) deficiency
Bulgarian	Омигапил малеат	Лечение на вродена мускулна дистрофия с мерозин (ламинин алфа 2) дефицит
Czech	Omigapil maleát	Léčba kongenitální muskulární dystrofie s deficiencí merosinu (laminin alfa 2)
Danish	Omigapil-maleat	Behandling af kongenit muskeldystrofi med merosin (laminin alpha2) -mangel
Dutch	Omigapil maleaat	Behandeling van congenitale spierdystrofie met merosinedeficiëntie (laminine-alfa-2-deficiëntie)
Estonian	Omigapiilmaleaat	Merosiinivaegusega (alfa-2 laminiin) kaasasündinud lihasdüstroofia ravi
Finnish	Omigapiilimaleaatti	Merosiinin (alfa-2-laminiinin) puutteesta johtuvan synnynnäisen lihasdystrofian hoito
French	Maléate d'omigapil	Traitement de la dystrophie musculaire congénitale avec déficit en mérosine (laminine alpha 2)
German	Omigapilmaleat	Behandlung der kongenitalen Muskeldystrophie mit Merosin (Laminin-alpha2)-Defizienz
Greek	Μηλεϊνική ομιγαπίλη	Θεραπεία της συγγενούς μυϊκής δυστροφίας με ανεπάρκεια μεροσίνης (λαμίνη Α2)
Hungarian	Omigapil maleát	Merosin (laminin alfa2) hiányos congenitalis izomdystrophiák kezelése
Italian	Omigapil maleato	Trattamento della distrofia muscolare congenita da deficit di merosina (laminina alfa-2)
Latvian	Omigapilmaleāts	Iedzimtas muskuļu distrofijas ar merozīna (laminīna-alfa 2) trūkumu ārstēšana
Lithuanian	Omigapilio maleatas	Įgimtos raumenų distrofijos, sąlygotos merozino (alfa 2 laminino) nepakankamumo, gydymas
Maltese	Omigapil maleate	Kura tad-distrofija muskolari konġenitali b'nuqqas ta' merosina (laminina alfa 2)
Polish	Maleinian omigapilu	Leczenie wrodzonej dystrofii mięśni z niedoborem merozyny (lamininy alfa 2)
Portuguese	Maleato de Omigapilo	Tratamento da distrofia muscular congénita com deficiência de merosina (laminina alfa 2)
Romanian	Maleat de omigapil	Tratamentul distrofiei musculare congenitale cu deficit de merozină (laminină alfa 2)
Slovak	Omigapilmaleát	Liečba vrodenej svalovej dystrofie s nedostatkom merozínu (laminin alfa 2)
Slovenian	Omigapilov maleat	Zdravljenje kongenitalne mišične distrofije zaradi pomanjkanja merozina (laminin alfa 2)

[1] At the time of designation

Language	Active Ingredient	Indication
Spanish	Maleato de omigapilo	Tratamiento de la distrofia muscular congénita con deficiencia de merosina (laminina alfa 2)
Swedish	Omigapil maleat	Behandling av kongenital muskeldystrofi med brist på merosin (laminin alfa-2)
Norwegian	Omigapilmaleat	Behandling av kongenital muskulær dystrofi med merosin (laminin-alfa2)-mangel
Icelandic	Ómigapíl maleat	Meðferð við meðfæddri vöðvarýrnun með merósín (laminín-alfa-2) skorti