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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Oleylphosphocholine for the treatment of leishmaniasis

On 23 April 2012, orphan designation (EU/3/12/964) was granted by the European Commission to Dafra Pharma International NV, Belgium, for oleylphosphocholine for the treatment of leishmaniasis.

What is leishmaniasis?

Leishmaniasis is a disease caused by infection with leishmania parasites and is transmitted to humans through the bite of sand flies. Following the bite of the sand fly, the parasite is taken up by cells of the body's immune system, multiplies within these cells and then spreads to other cells and tissues in the body.

The most common form of the disease is cutaneous (skin) leishmaniasis, which involves raised, scaly lesions appearing on the skin in various parts of the body. The most severe form of the disease is visceral leishmaniasis, in which the parasites have migrated to the major organs, causing the enlargement of the liver, spleen and lymph nodes, and symptoms such as high fever, chills, weight loss and tiredness. The disease is found more frequently in tropical regions of the world. In Europe, leishmaniasis mostly affects the southern parts of the continent but is increasingly seen in people with weakened immune systems, such as patients with HIV or patients taking immunosuppressive medicines for cancer or transplantation.

Leishmaniasis is a long-term debilitating and life-threatening disease due to weight loss, organ enlargement and recurrent episodes of fever.

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

At the time of designation, leishmaniasis affected approximately 0.01 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 500 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 on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

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
An agency of the European Union

What treatments are available?

At the time of designation, medicines including pentavalent antimonials, amphotericin B, miltefosine, ketoconazole and pentamidine were authorised in various EU countries for the treatment of leishmaniasis.

The sponsor has provided sufficient information to show that oleylphosphocholine might be of significant benefit for patients with leishmaniasis because early studies in experimental models show that it might have a greater effect than miltefosine in clearing the parasites from body's organs. In addition, this medicine will be developed as a topical (skin) as well as a systemic formulation (to be given as treatment throughout the body).The availability of a topical formulation could result in a major contribution to patient care as it is a new route of administration in the treatment of this condition. Finally, the way oleylphosphocholine works and the fact that it will be able to be given into a vein may help with the development of combination treatments with other medicines. These assumptions will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

The way in which oleylphosphocholine may work in leishmaniasis is unclear, but it is expected to work in a similar way to another anti-leishmaniasis medicine, miltefosine, as the two medicines share a similar structure. Miltefosine works by blocking the action of a group of enzymes known as 'AKT' that are involved in cell division. Oleylphosphocholine is also expected to block cell division, leading to the death of the parasites and curing the patients of the disease.

What is the stage of development of this medicine?

The effects of oleylphosphocholine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with oleylphosphocholine in patients with leishmaniasis had been started.

At the time of submission, oleylphosphocholine was not authorised anywhere in the EU for leishmaniasis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 January 2012 recommending the granting of this designation.

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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:

Dafra Pharma International NV
Slachthuisstraat 30/7
2300 Turnhout
Belgium
Telephone: +32 14 61 78 20
Telefax: +32 14 61 78 59
E-mail: info@dafra.be

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 substance	Indication
English	Oleylphosphocholine	Treatment of leishmaniasis
Bulgarian	Олеилфосфохолин	Лечение на лайшманиоза
Czech	Oleylphosphocholine	Léčba leishmaniózy
Danish	Oleylphosphocholin	Behandling af leishmaniasis
Dutch	Oleylphosphocholine	Behandeling van leishmaniasis
Estonian	Oleilfosfokoliin	Leišmanioosi ravi
Finnish	Oleylifosfokoliini	Leishmaniaasin hoito
French	Oleylphosphocholine	Traitement de la leishmaniose
German	Oleylphosphocholine	Therapie der Leishmaniose
Greek	Ολεϊλφωσφοχολίνη	Θεραπεία της λεϊσμανίασης
Hungarian	Oleylphosphocholine	Leishmaniasis kezelése
Italian	Oleilfosfocolina	Trattamento della leishmaniosi
Latvian	Oleilfosfoholīns	Leišmaniozes ārstēšana
Lithuanian	Oleilfosfocholinas	Leišmaniozės gydymas
Maltese	Oleylphosphocholine	Kura ta' leishmaniasis
Polish	Oleilofosfocholina	Leczenie leiszmaniozy
Portuguese	Oleilfosfocolina	Tratamento de leishmaniose
Romanian	Oleilfosfocolină	Tratamentul leishmaniozei
Slovak	Oleylfosfocholín	Liečba leishmaniózy
Slovenian	Oleilphosfoholin	Zdravljenje leishmaniaze
Spanish	Oleilfosfocolina	Tratamiento de la leishmaniasis
Swedish	Oleylfosfokolin	Behandling av leishmaniasis
Norwegian	Oleylfosfokolin	Behandling av leishmaniasis
Icelandic	Óleylfosfókólín	Meðferð blökkusóttar

[1] At the time of designation