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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Miltefosine for the treatment of *Acanthamoeba* keratitis

First publication	29 June 2005
Rev.1: transfer of sponsorship	18 August 2008
Rev.2: transfer of sponsorship	12 August 2009
Rev.3: administrative update	25 October 2012
Rev.4: administrative update	10 December 2013

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 27 May 2005, orphan designation (EU/3/05/282) was granted by the European Commission to Obwaller Forschungs- und Entwicklungs GmbH, Austria, for miltefosine for the treatment of *Acanthamoeba* keratitis.

The sponsorship was transferred to Laboratoires Théa, France, in July 2007 and subsequently to Orphanidis Pharma Research GmbH, Austria, in December 2008.

What is *Acanthamoeba* keratitis?

Acanthamoeba are organisms consisting of one single cell that can cause an eye disease. These organisms live and survive in many different places such as air, soil and water environments. From these sources, they can gain access to contact lens solutions and contact lenses. Tap water used to clean lenses is a common source of such an infection. The *Acanthamoeba* may be on the lens or in the cleaning solutions. If lenses are left in the eyes for long periods of time, these amoebae can multiply and cause damage to the eye (tears and ulcers).

Acanthamoeba keratitis is a chronically debilitating condition and may result in loss of sight in the infected eye.

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

At the time of designation, *Acanthamoeba* keratitis affected not more than 0.1 in 10,000 people in 10,000 people in the European Union (EU). This is equivalent to a total of not more than 4,700 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?

Although it is not yet fully understood how miltefosine acts in A*canthamoeba* keratitis, it seems that it might destroy the walls of the amoeba cells. Therefore, it might eradicate the amoeba (amoebicidal).

What is the stage of development of this medicine?

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

The medicinal product was not marketed anywhere worldwide for *Acanthamoeba* keratitis 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 7 April 2005 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.

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).

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	Miltefosine	Treatment of Acanthamoeba keratitis
Bulgarian	Милтефозин	Лечение на кератит, причинен от акантамеба
Czech	Miltefosine	Léčba acanthamoebová keratitida
Danish	Miltefosin	Behandling af acanthamøbe Keratoconjunctivitis
Dutch	Miltefosine	Acanthamoeba keratitis
Estonian	Miltefosiin	Acanthamoeba keratiidi ravi
Finnish	Miltefosiini	Akantamebakeratiitin hoito
French	Miltéfosine	Traitement de la kératite causée par l'acanthamoeba
German	Miltefosin	Acanthamoeba-Keratitis
Greek	Μιλτεφοσίνη	Θεραπεία της κερατίτιδας από Acanthamoeba
Hungarian	Miltefosin	Acanthamoeba keratitis kezelése
Italian	Miltefosina	Trattamento della cheratite da Acanthamoeba
Latvian	Miltefozīns	Akantamebiāzes keratīta ārstēšana
Lithuanian	Miltefozinas	Akantamebinio keratito gydymas
Maltese	Miltefosine	Kura tal-keratite ikkawżata mill-Acanthamoeba
Polish	Miltefozyna	Zapalenie rogówki wywołane przez Acanthamoeba
Portuguese	Miltefosina	Tratamento da ceratite por acanthamoeba
Romanian	Miltefosină	Tratamentul keratitei produse de Acanthamoeba
Slovak	Miltefosín	Liečba acanthamoeba keratitis
Slovenian	Miltefosin	Zdravljenje akatamebnega keratitisa
Spanish	Miltefosina	Tratamiento de la queratitis por acanthamoeba
Swedish	Miltefosin	Bahandling av acanthamöba-keratit
Norwegian	Miltefosin	Acantamøbe-keratitt
Icelandic	Miltefósín	Meðferð á þyrniamöbu glærubólgu

[1] At the time of transfer of sponsorship