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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Paromomycin sulfate for the treatment of visceral leishmaniasis

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in March 2011 on request of the sponsor.

On 11 April 2005, orphan designation (EU/3/05/271) was granted by the European Commission to OneWorld Health, United Kingdom, for paromomycin sulfate for the treatment of visceral leishmaniasis.

The sponsorship was transferred to Sestria Ltd, United Kingdom, in November 2009.

What is visceral leishmaniasis?

Leishmania are parasites that consist of one single cell, which are transmitted to humans through the bite of sand flies, mostly in the southern part of Europe and other parts of the world. Following the bite of the sandfly, the parasite is put in the skin where it will be taken up by specific cells of the body's defence system. The parasite will then multiply in that type of cell and spread through the body causing infections. Visceral leishmaniasis, the most severe form of leishmaniasis, will create infections in the cavities of the body where the major organs are located (most often in the abdomen). Patients having already an ongoing disease that suppress their own defense system (such as HIV infected patients) will be more sensitive to get infected by the *Leishmania*.

Visceral leishmaniasis is a chronically debilitating and life-threatening condition.

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

At the time of designation, visceral leishmaniasis affected approximately 0.1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 4,600 people, and is below the threshold 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 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

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What treatments are available?

Several products had been authorised for the condition in the Community at the time of submission of the application for orphan designation. Although a significant percentage of patients respond to the initial therapy, many of the immuno-suppressed patients tend to get the *leishmania* infection back and will not respond well to therapy anymore with the available products.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that the medicinal product might be of potential significant benefit for the treatment of visceral leishmaniasis, particularly because it might improve the long-term outcome of the patients. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Paromomycin sulfate is an antibiotic (drugs that fight bacterial infections) belonging to a certain class, the so-called aminoglycosides, which also has shown activity against parasitic infections.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with visceral leishmaniasis were ongoing.

The medicinal product was not marketed anywhere worldwide for visceral leishmaniais 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 3 March 2005 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 European Union) 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	Paromomycin sulphate	Treatment of visceral leishmaniasis
Bulgarian	Паромомицин сулфат	Лечение на висцерална лайшманиоза
Czech	Paromomycin sulfát	Léčba viscerální leishmaniózy
Danish	Paromomycinsulfat	Behandling af visceral leishmaniasis
Dutch	Paromomycinesulfaat	Behandeling van viscerale leishmaniasis
Estonian	Paromomütsiinsulfaat	Vistseraalse leišmanioosi ravi
Finnish	Paromomysiinisulfaatti	Viskeraalisen leishmaniaasin hoito
French	Sulfate de paromomycine	Traitement de la leishmaniose viscérale
German	Paromomycinsulphat	Therapie der viszeralen Leishmaniose
Greek	Θειϊκό άλας παρομομυκίνης	Θεραπεία της σπλαχνικής λεϊσμανίασης
Hungarian	Paromomicin-szulfát	Visceralis leishmaniasis kezelésére
Italian	Paromomicina solfato	Trattamento della leishmaniosi viscerale
Latvian	Paromomicīna sulfāts	Viscerālās leišmaniozes ārstēšana
Lithuanian	Paromomicino sulfatas	Visceralinės leišmaniozės gydymas
Maltese	Paromomycin sulphate	Kura ta' *leishmaniasis* tal-vixxri
Polish	Paromomycyny siarczan	Leczenie leiszmaniozy trzewnej.
Portuguese	Sulfato de paromomicina	Tratamento de leishmaniose visceral
Romanian	Sulfat de paromomicină	Tratamentul leishmaniozei viscerale
Slovak	Paromomycín sulfát	Liečba viscerálnej leišmaniózy
Slovenian	Paromomicinijev sulfat	Zdravljenje visceralne leishmanioze
Spanish	Sulfato de paromomicina	Tratamiento de la leishmaniasis visceral
Swedish	Paromomycinsulfat	Behandling av visceral leishmaniasis
Norwegian	Paromomycinsulfat	Behandling av visceral leishmaniasis
Icelandic	Parómómysínsúlfat	Meðferð blökkusóttar

[1] At the time of transfer of sponsorship