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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tretazicar for the treatment of visceral leishmaniasis

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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 4 February 2008, orphan designation EU/3/08/529 was granted by the European Commission to Morvus Technology Limited, United Kingdom, for tretazicar for the treatment of visceral leishmaniasis.

What is visceral leishmaniasis?

Leishmania are parasites, which are transmitted to humans through the bite of sand flies. In Europe the disease is found mostly in the southern parts of Europe; it can also be found much more frequently in tropical regions of the World. Following the bite of the sandfly, the parasite is introduced into the skin where it will be taken up by some cells of the body's defence system. The parasite will then multiply in these cells and spread through the body and cause infection. The most severe form of leishmaniasis, the so called visceral leishmaniasis, is caused by parasite infections in the cavities of the body where the major organs are located (most often the abdomen). Patients having already another disease (such as HIV) that suppresses their own defense system are more sensitive to infection 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 was equivalent to a total of around 5,000 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 502,800,000 (Eurostat 2008).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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?

Several medicinal products were authorised for the condition in the Community at the time of submission of the application for orphan designation. Although a significant proportion of patients respond to the initial therapy, many of the immune-suppressed patients tend to get the leishmania infection back and will then not respond well to the therapy.

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 may 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?

Tretazicar is a product that belongs to a family of products used in the treatment of cancer because of its capacity to kill cells. Tetrazicar is active only after it is transformed inside cells. This is done by an enzyme that is not found in human cells, therefore the damaging activity of tretazicar cannot be seen in humans. However, the enzyme that activates tretazicar is present in leishmania cells where the product can be transformed and be active. Tretazicar kills infected cells by binding to the genetic material (DNA) of the cells and creating bonds that disrupt the genetic material and its function, finally resulting in the death of the infected cell.

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 initiated.

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 10 January 2008 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:

Morvus Technology Limited
Llanvetherine Court
Llanvetherine
Abergavenny NP7 8NL
United Kingdom
Telephone: +44 (0)1558 668111
E-mail: info@morvus.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	Tretazicar	Treatment of visceral leishmaniasis
Bulgarian	Третазикар	Лечение на висцерална лайшманиоза
Czech	Tretazicar	Léčba viscerální leishmaniózy
Danish	Tretazicar	Behandling af visceral leishmaniasis
Dutch	Tretazicar	Behandeling van viscerale leishmaniasis
Estonian	Tretasikaar	Vistseraalse leišmanioosi ravi
Finnish	Tretatsikaari	Viskeraalisen leishmaniaasin hoito
French	Trétazicar	Traitement de la leishmaniose viscérale
German	Tretazicar	Therapie der viszeralen Leishmaniose
Greek	Τρεταζικάρη	Θεραπεία της σπλαχνικής λεϊσμανίασης
Hungarian	Tretazicar	Visceralis leishmaniasis kezelése
Italian	Tretazicar	Trattamento della leishmaniosi viscerale
Latvian	Tretazikars	Viscerālās leišmaniozes ārstēšana
Lithuanian	Tretazikaras	Visceralinės leišmaniozės gydymas
Maltese	Tretazicar	Kura ta' *leishmaniasis* tal-vixxri
Polish	Tretazicar	Leczenie leiszmaniozy trzewnej.
Portuguese	Tretazicar	Tratamento de leishmaniose visceral
Romanian	Tretazicar	Tratamentul leishmaniozei viscerale
Slovak	Tretazikar	Liečba viscerálnej leišmaniózy
Slovenian	Tretazicar	Zdravljenje visceralne leishmanioze
Spanish	Tretazicar	Tratamiento de la leishmaniasis visceral
Swedish	Tretazicar	Behandling av visceral leishmaniasis
Norwegian	Tretazicar	Behandling av visceral leishmaniasis
Icelandic	Tretazíkar	Meðferð blökkusóttar

[1] At the time of designation