



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

11 December 2012
EMA/COMP/684363/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Melarsoprol for treatment of African trypanosomiasis

On 8 November 2012, orphan designation (EU/3/12/1068) was granted by the European Commission to Pr. Peter Kennedy, United Kingdom, for melarsoprol for treatment of African trypanosomiasis.

What is African trypanosomiasis?

African trypanosomiasis, also known as sleeping sickness, is an infectious disease caused by *Trypanosoma* parasites. It is transmitted to humans through the bite of an infected tsetse fly. The disease is widespread in sub-Saharan African countries. In Europe, the disease mainly affects travellers returning from these areas.

The symptoms of the disease occur in two stages. In the first stage, following infection, a red sore appears at the site of the tsetse fly bite, and within a few weeks the person experiences intermittent fever, swollen lymph glands, pain in muscles and joints, headache and malaise (feeling unwell). In the second advanced stage, the parasites invade the central nervous system, causing confusion, coordination problems, seizures, and disruption of sleep patterns.

African trypanosomiasis is a long-term debilitating disease that is fatal if left untreated.

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

At the time of designation, African trypanosomiasis affected approximately 0.00004 in 10,000 people in the European Union (EU)*. This is equivalent to a total of 2 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?

At the time of designation, several medicines were available to treat African trypanosomiasis, including pentamidine, nifurtimox, eflornithine, melarsoprol and suramin. All these medicines were given by

*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).



injection into a vein. Melarsoprol given by injection is the main treatment for the disease that has progressed to the central nervous system.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with African trypanosomiasis because it is a new formulation of melarsoprol that is taken by mouth, which is expected to be less toxic than melarsoprol given by injection. This assumption 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?

Melarsoprol is a compound containing arsenic that has been used in injectable form for the treatment of African trypanosomiasis for several decades. The way that melarsoprol works is unclear, but it is thought to kill Trypanosoma parasites by disrupting their energy-production mechanisms. As a result of the diminished energy production, the parasite eventually dies. However, melarsoprol injections are highly toxic and may themselves lead to death in up to 5% of patients.

This medicine is an oral formulation designed to make treatment safer. It is made of melarsoprol contained into molecules called cyclodextrins, which are expected to carry melarsoprol through to the brain with fewer side effects. Cyclodextrins are used in several medicines to improve delivery of active substances and to improve safety.

What is the stage of development of this medicine?

The effects of the cyclodextrin formulation of melarsoprol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the cyclodextrin formulation of melarsoprol in patients with African trypanosomiasis had been started.

At the time of submission, melarsoprol was not authorised anywhere in the EU for African trypanosomiasis 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 5 October 2012 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:

Pr. Peter Kennedy
Institute of Neurological Sciences
Southern General Hospital
Glasgow G51 4TF
United Kingdom
Telephone: +44 141 201 2474
Telefax: +44 141 201 2993
E-mail: Peter.Kennedy@glasgow.ac.uk

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 languages¹, Norwegian and Icelandic

Language	Active substance	Indication
English	Melarsoprol	Treatment of African trypanosomiasis
Bulgarian	Меларсопрол	Лечение на африканска трипанозомиаза
Czech	Melarsoprol	Léčba africké trypanosomiázy
Danish	Melarsoprol	Behandling af trypanosomiasis
Dutch	Melarsoprol	Behandeling van Afrikaanse trypanosomiasis
Estonian	Melarsoprol	Ravi trypanosomiasis
Finnish	Melarsoprol	Afrikkalaisen trypanosomiaasin hoito
French	Mélarso­prol	Traitement de la trypanosomiose
German	Melarsoprol	Die Behandlung der Schlafkrankheit
Greek	Μελαρσοπρόλη	Θεραπεία της αφρικανικής τρυπανοσωμίας
Hungarian	Melarsoprol	Afrikai trypanosomiasis kezelése
Italian	Melarsoprol	Trattamento di tripanosomiasi
Latvian	Melarsoprols	Āfrikas tripanosomiāzes ārstēšana
Lithuanian	Melarsoprolis	Afrikietiškos tripanosomiazės gydymas
Maltese	Melarsoprol	Kura tat-tripanosomijasi Afrikana
Polish	Melarsoprol	Leczenie trypanosomatozy afrykańskiej
Portuguese	Melarsoprol	Tratamento da tripanosomíase africana
Romanian	Melarsoprol	Tratamentul tripanosomiazelor africane
Slovak	Melarsoprol	Liečba africkéj trypanozomiázy
Slovenian	Melarsoprol	Zdravljenje afriške tripanosomioze
Spanish	Melarsoprol	El tratamiento de la tripanosomiasis africana
Swedish	Melarsoprol	Behandling av afrikansk trypanosomiasis
Norwegian	Melarsoprol	Behandling av afrikansk trypanosomiasis
Icelandic	Melarsópról	Meðferð manna trypanosomiasis

¹ At the time of designation