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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of miltefosine for the treatment of cutaneous T-cell lymphoma

On 22 September 2008, orphan designation (EU/3/08/567) was granted by the European Commission to ExperGen Drug Development GmbH, Austria, for miltefosine for the treatment of cutaneous T-cell lymphoma.

What is cutaneous T-cell lymphoma?

Cutaneous T-cell lymphoma (CTCL) is a cancer of the lymphatic system, a complex system in the body that works as part of the body's immune system to help to fight infections. In CTCL there is uncontrolled growth of the T-lymphocytes (T-cells), a type of white blood cell found in the lymphatic system. The cancerous T-cells appear in the skin, causing lesions (rashes, plaques and tumours). CTCL usually happens in people aged between 40 and 60 years. In many cases, the disease is long-lasting, with survival for more than 10 to 20 years being common. However, it can be a serious and life-threatening disease, with a severe impact on quality of life.

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

At the time of designation CTCL affected approximately 1.5 in 10,000 people in the European Union (EU) *. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 75,000 people.

What treatments are available?

At the time of submission of the application for orphan designation, several products were authorised for the treatment of CTCL within the EU. Treatments for CTCL can be divided into local (affecting the skin only) and systemic (affecting the whole body):

- local treatments include medicines applied to the skin, ultraviolet light and X-rays;
- systemic treatments include glucocorticoids (a group of steroid medicines that reduce the division of T-cells), cytotoxic medicines (medicines that kill cells, including cancer cells), interferon alfa (a medicine that helps the immune system to fight against the cancer cells) and photopheresis. Photopheresis is a technique in which blood is temporarily removed from the body to be treated using light. The red cells and plasma are then returned to the patient while the white cells, including the affected T-lymphocytes, are treated with a substance outside the body. This substance becomes activated when exposed to light and kills tumour cells. The treated white cells are then returned to the patient.

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

The sponsor has provided sufficient information to show that miltefosine might be of significant benefit for patients because it might be more effective than existing treatments. This assumption will have to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

The exact way that miltefosine works is not known. It is thought to interact with the cell surface, starting a chain of events in leading to the cell's death. The sponsor plans to develop a form of miltefosine that will be applied directly onto the skin lesions caused by CTCL.

What is the stage of development of this medicine?

The effects of miltefosine have been evaluated in experimental models.

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

Miltefosine is authorised in Germany as an orphan medicine for the treatment of visceral leishmaniasis (a disease caused by infection with a parasite). A form that can be administered directly into the skin is also authorised in several EU Member States for the treatment of cutaneous metastases of breast cancer (when breast cancer tumours have spread to the skin).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 July 2008 recommending the granting of the above-mentioned.

Opinions on orphan medicinal products designations are based on the following 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 five 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 of a product is necessary before it can be granted a marketing authorisation.

For more information:

Sponsor's contact details:
ExperGen Drug Development GmbH
Hernalser Hauptstraße 24/16
1170 Vienna
Austria
Telephone: + 43 1 40 59 786
Telefax: + 43 1 40 59 785
E-mail: p.meng@aon.at

Patients' associations contact points:

Ligue Nationale Contre le Cancer

14 Rue Corvisart

75013 Paris

France

Telephone: +33 1 53 55 24 00

Telefax: +33 1 43 36 91 10

E-mail: ligue@ligue-cancer.net

Deutsche Krebshilfe e. V.

Buschstr. 32

53113 Bonn

Germany

Telephone: +49 2 287 29 900

Telefax: +49 2 287 29 90 11

E-mail: deutsche@krebshilfe.de

Macmillan Cancer Support (merged with CancerBACUP)

3 Bath Place

Rivington Street

London

EC2A 3JR

United Kingdom

Telephone: +44 20 76 96 90 03

Switchboard open during office hours, Mon–Fri, 9am–Noon and 2pm–4.45pm

Telefax: +44 20 76 96 90 02

**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Miltefosine	Treatment of cutaneous T-cell lymphoma
Bulgarian	Милтефозин	Лечение на кожен Т-клетъчен лимфом
Czech	Miltefosine	Léčba kožního T-lymfomu
Danish	Miltefosin	Behandling af kutant T-celle-lymfom
Dutch	Miltefosine	Behandeling van cutaan T-cel-lymfoom
Estonian	Miltefosiin	Kutaanse T-rakulise lümfoomi ravi
Finnish	Miltefosiini	Ihon T-solulymfooman hoito
French	Miltéfosine	Traitement des lymphomes cutanés à cellules T
German	Miltefosin	Behandlung von kutanem T-Zell- Lymphom
Greek	Μιλτεφοσίνη	Θεραπεία του δερματικού λεμφώματος Τα κυττάρων
Hungarian	Miltefosin	T-sejtes cutan lymphoma kezelése
Italian	Miltefosina	Trattamento del linfoma cutaneo a cellule T
Latvian	Miltefozīns	Ādas T-šūnu limfomas ārstēšana
Lithuanian	Miltefozinas	Odos T ląstelių limfomos gydymas
Maltese	Miltefosine	Kura tal-limfoma taċ-ċelluli tat-tip T tal-ġilda
Polish	Miltefozyna	Leczenie chłoniaka skórniego T-komórkowego
Portuguese	Miltefosina	Tratamento do linfoma cutâneo de células T
Romanian	Miltefosină	Tratamentul limfomului cutanat cu celule T
Slovak	Miltefosín	Liečba kutánneho T-bunkového lymfómu
Slovenian	Miltefosin	Zdravljenje kožnega T-celičnega limfoma
Spanish	Miltefosina	Tratamiento del linfoma cutáneo de células T
Swedish	Miltefosin	Behandling av kutant T-cellslymfom
Norwegian	Miltefosin	Behandling av kutant T-cellelymfom
Icelandic	Miltefösín	Meðferð T-eitilfrumukrabbameins í húð