



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fenretinide for the treatment of cutaneous T-cell lymphoma

On 21 March 2016, orphan designation (EU/3/16/1630) was granted by the European Commission to Clinipace GmbH, Germany, for fenretinide 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 network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream.

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) which can be itchy and painful.

CTCL usually happens in people aged between 40 and 60 years. In many cases, patients survive a long time with the disease; however, in some cases the disease can be serious and life threatening because it can develop into more aggressive forms of cancer and may have a large impact on quality of life, particularly because the skin lesions can cause disfigurement.

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

At the time of designation, CTCL affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 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).

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).



What treatments are available?

At the time of designation, several products were authorised for the treatment of CTCL within the EU. Treatments for CTCL can be divided into topical (applied to the skin) and systemic (affecting the whole body):

- topical treatments included topical corticosteroids, the topical cancer medicine carmustine and ultraviolet light;
- systemic treatments included cytotoxic medicines (medicines that kill cells that are dividing, such as cancer cells) and interferon alfa (a medicine that helps the immune system to fight against the cancer cells).

The sponsor has provided sufficient information to show that fenretinide might be of significant benefit for patients with CTCL because early studies show that patients whose disease had come back after previous treatment or did not respond to previous treatment responded to treatment with this medicine. 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?

The exact mechanism of action of fenretinide is not fully understood but it is thought to cause death of cancer cells through different mechanisms such as the production of ceramide and reactive oxygen species (ROS, toxic molecules containing oxygen) which induce cancer cell death.

What is the stage of development of this medicine?

The effects of fenretinide have been evaluated in experimental models.

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

At the time of submission, fenretinide was not authorised anywhere in the EU for CTCL. Orphan designation of the medicine had been granted in the United States for cutaneous T-cell lymphoma and for peripheral T-cell lymphoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 February 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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 ingredient	Indication
English	Fenretinide	Treatment of cutaneous T-cell lymphoma
Bulgarian	Фенретинид	Лечение на кожен Т-клетъчен лимфом
Croatian	Fenretinid	Liječenje kožnog limfoma T-stanica
Czech	Fenretinid	Léčba kožního T-lymfomu
Danish	Fenretinid	Behandling af kutant T-celle-lymfom
Dutch	Fenretinide	Behandeling van cutaan T-cel-lymfoom
Estonian	Fenretiniid	Kutaanse T-rakulise lümfoomi ravi
Finnish	Fenretinidi	Ihon T-solulymfooman hoito
French	Fenrétinide	Traitement des lymphomes cutanés à cellules T
German	Fenretinide	Behandlung von kutanem T-Zell- Lymphom
Greek	Φενρετινίδη	Θεραπεία του δερματικού λεμφώματος Τ-κυττάρων
Hungarian	Fenretinid	Kután T-sejtes lymphoma kezelése
Italian	Fenretinide	Trattamento del linfoma cutaneo a cellule T
Latvian	Fenretinīds	Ādas T-šūnu limfomas ārstēšana
Lithuanian	Fenretinidas	Odos T ląstelių limfomos gydymas
Maltese	Fenretinide	Kura tal-linfoma taċ-ċelluli tat-tip T tal-ġilda
Polish	Fenretynid	Leczenie chłoniaka skórniego T-komórkowego
Portuguese	Fenretinida	Tratamento do linfoma cutâneo de células T
Romanian	Fenretinidă	Tratamentul limfomului cutanat cu celule T
Slovak	Fenretinid	Liečba kutáneho T-bunkového lymfómu
Slovenian	Fenretinid	Zdravljenje kožnega T-celičnega limfoma
Spanish	Fenretinida	Tratamiento del linfoma cutáneo de células T
Swedish	Fenretinid	Behandling av kutant T-cellslymfom
Norwegian	Fenretinid	Behandling av kutant T-cellelymfom
Icelandic	Fenretíníð	Meðferð T-eitilfrumukrabbameins í húð

¹ At the time of designation