



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

7 November 2016
EMA/622420/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fenretinide for the treatment of peripheral T-cell lymphoma

On 14 October 2016, orphan designation (EU/3/16/1751) was granted by the European Commission to Clinipace GmbH, Germany, for fenretinide for the treatment of peripheral T-cell lymphoma.

What is peripheral T-cell lymphoma?

Peripheral T-cell lymphoma 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 peripheral T-cell lymphoma there is uncontrolled growth of T lymphocytes (T cells), a type of white blood cell found in the lymphatic system. Peripheral T-cell lymphomas include types that mainly occur in the lymph nodes (primary nodal) and types that occur mainly outside the lymph nodes (primary extranodal).

The symptoms of the disease vary according to the type of lymphoma, but the first sign may be a lump in the neck, under the arm or in the groin area, which is caused by an enlarged lymph node. The lymphoma may also affect other organs in the body such as the bone marrow, liver and the skin.

Peripheral T-cell lymphoma is a long-term debilitating and life-threatening condition because in most cases the disease does not respond well to therapy, usually comes back within one year and is associated with poor overall survival.

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

At the time of designation, peripheral T-cell lymphoma affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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).

What treatments are available?

At the time of designation, there were no specific treatments for peripheral T-cell lymphoma, but the disease was treated in the same way as the broader class of lymphomas known as non-Hodgkin's

*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).



lymphomas, for which several medicines were authorised in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that fenretinide might be of significant benefit for patients with peripheral T-cell lymphoma because early results suggested it might produce a response in patients who had not responded to previous treatments or whose disease had come back after treatment. 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 certain toxic ceramides 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 fenretinide in patients with peripheral T-cell lymphoma were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 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 peripheral T-cell lymphoma
Bulgarian	Фенретинид	Лечение на периферен Т-клетъчен лимфом
Croatian	Fenretinid	Liječenje perifernog limfoma T-stanica
Czech	Fenretinid	Léčba periferních T-lymfomů
Danish	Fenretinid	Behandling af perifer T-celle lymfom
Dutch	Fenretinide	Behandeling van perifere T-cel lymfomen
Estonian	Fenretiniid	Perifeerse T-rakulise lümfoomi ravi
Finnish	Fenretinidi	Perifeerisen T-solulymfooman hoito
French	Fenrétinide	Traitement du lymphome périphérique à cellules T
German	Fenretinide	Behandlung des peripheren T-Zell-Lymphoms
Greek	Φενρετινίδη	Θεραπεία του λεμφώματος περιφερικών κυττάρων T
Hungarian	Fenretinid	Perifériás T-sejtes lymphoma kezelése
Italian	Fenretinide	Tattamento del linfoma periferico a cellule T
Latvian	Fenretinīds	Perifēriskās T-šūnu limfomas ārstēšana
Lithuanian	Fenretinidas	Periferinės T-ląstelių limfomos gydymas
Maltese	Fenretinide	Kura tal-linfoma taċ-ċelloli T periferali
Polish	Fenretynid	Leczenie obwodowego chłoniaka T-komórkowego
Portuguese	Fenretinida	Tratamento do linfoma periférico das células T
Romanian	Fenretinidă	Tratamentul limfomului periferic cu celule T
Slovak	Fenretinid	Liečba periférneho T-bunkového lymfómu
Slovenian	Fenretinid	Zdravljenje perifernega limfoma celic T
Spanish	Fenretinida	Tratamiento del linfoma periférico de células T
Swedish	Fenretinid	Behandling av perifert T-cellslymfom
Norwegian	Fenretinid	Behandling av perifert T-celle-lymfom
Icelandic	Fenretíníð	Meðferð við útlægu T-eitilfrumukrabbameini

¹ At the time of designation