23 March 2011
EMA/COMP/96682/2008 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fenretinide for the treatment of primary malignant bone tumours

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2011 on request of the Sponsor.

On 26 January 2007, orphan designation (EU/3/06/426) was granted by the European Commission to Cancer Research UK, United Kingdom, for fenretinide for the treatment of primary malignant bone tumours.

What are primary malignant bone tumours?

Tumours that arise in the bone tissues are known as primary malignant bone tumours. Primary malignant bone tumours are classified by the type of tissue they originate, the most common being bone sarcomas. They can occur in any bone of the body and they spread almost exclusively through the blood. There are many types of primary malignant bone tumours, which tend to behave differently. Having a primary malignant bone tumour is a serious condition, potentially debilitating and life-threatening.

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

At the time of designation, primary malignant bone tumours affected less than 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 46,000 people, and is below the threshold 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?

Surgery is currently the main choice of therapy for primary malignant bone tumours. It is usually combined with chemotherapy (using drugs to kill cancer cells) prior to or after surgery. Several

[*]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 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
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products were authorised for the condition in some Member States in the Community at the time of submission of the application for orphan drug designation.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that fenretinide might be of potential significant benefit for the treatment of primary malignant bone tumours mainly because it might improve the long-term outcome of the patients. This 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?

Fenretinide is structurally related to vitamin A. The exact mechanism of action of fenretidine is not fully understood but it is thought that it binds to, and kills tumour cells in primary malignant bone tumours.

What is the stage of development of this medicine?

The effects of fenretinide were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with primary malignant bone tumours were initiated. However some clinical trials were planned, at the time of submission.

Fenretinide was not authorised anywhere worldwide for treatment of primary malignant bone tumours 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 6 December 2006 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:

Cancer Research UK
61 Lincoln's Inn Fields
London, WC2A 3PX
United Kingdom
Telephone: 44 207 121 69 38
Telefax: 44 207 121 69 02
E-mail: sally.burtles@cancer.org.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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Fenretinide	Treatment of primary malignant bone tumours
Bulgarian	Фенретинид	Лечение на първични малигнени костни тумори
Czech	Fenretinid	Léčba primárních kostních nádorů
Danish	Fenretinid	Behandling af primære maligne knogletumorer
Dutch	Fenretinide	Behandeling van primair kwaadaardige bottumoren
Estonian	Fenretiniid	Primaarsete pahaloomuliste luukasvajate ravi
Finnish	Fenretinidi	Primaarisen pahanlaatuisen luukasvaimen hoito
French	Fenrétinide	Traitement des tumeurs malignes osseuses primaires
German	Fenretinide	Behandlung von primär malignen Knochentumoren
Greek	Fenretinide	Θεραπεία πρωτοπαθών κακοήθων όγκων των οστών
Hungarian	Fenretinid	Rosszindulatú primer csontdaganatok kezelése
Italian	Fenretinide	Trattamento di tumori primari maligni ossei
Latvian	Fenretinīds	Primāru ļaundabīgu kaulu audzēju ārstēšana
Lithuanian	Fenretinidas	Pirminių piktybinių kaulų auglių gydymas
Polish	Fenretynid	Leczenie pierwotnych złośliwych guzów kości
Portuguese	Fenretinida	Tratamento do tumor ósseo maligno primário
Romanian	Fenretinidă	Tratamentul tumorilor maligne primare osoase
Slovak	Fenretinid	Liečba primárneho malígneho tumoru kostí
Slovenian	Fenretinid	Zdravljenje primarnih malignih kostnih tumorjev
Spanish	Fenretinida	Tratamiento de los tumores malignos primarios de hueso
Swedish	Fenretinid	Behandling av primära maligna ben tumörer
Norwegian	Fenretinid	Behandling av primære maligne bentumorer
Icelandic	Fenretíníð	Meðferð við illkynja beinæxlum

[1] At the time of designation