



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 March 2011
EMA/COMP/96616/2008 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fenretinide for the treatment of soft tissue sarcoma

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2011 on request of the Sponsor.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 30 January 2007, orphan designation (EU/3/06/427) was granted by the European Commission to Cancer Research UK, United Kingdom, for fenretinide for the treatment of soft tissue sarcoma.

What is soft tissue sarcoma?

Soft tissue sarcomas are cancers of the supporting tissues of the body. They can occur in muscle, fat, blood vessels or in any of the other tissues that support, surround and protect the organs of the body. There are many types of soft tissue sarcoma. The cause of soft tissue sarcoma is largely unknown. Soft tissue sarcoma 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, soft tissue sarcoma affected less than 2.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 115,000 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 early stage soft tissue sarcomas. For larger sarcomas, and where it is thought there is a possibility of cancer cells being left behind, after a surgical intervention, radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) and chemotherapy (using drugs to kill cancer cells) are usually used. Several products for treatment of soft

*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.



tissue sarcoma 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 soft tissue sarcoma 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 fenretinide is not fully understood but it is thought that it binds to, and kills tumour cells in soft tissue sarcomas.

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 soft tissue sarcoma were initiated.

Fenretinide was not authorised anywhere worldwide for treatment of soft tissue sarcoma 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:

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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 soft tissue sarcoma
Bulgarian	Фенретинид	Лечение на сарком на меките тъкани
Czech	Fenretinid	Léčba sarkomu měkkých tkání
Danish	Fenretinid	Behandling af bløddelssarkom
Dutch	Fenretinide	Behandeling weke delen sarcoom
Estonian	Fenretiniid	Pehmeete kudede sarkoomi ravi
Finnish	Fenretinidi	Pehmytkudossarkooman hoito
French	Fenrétinide	Traitement des sarcomes des tissus mous
German	Fenretinide	Behandlung des Weichteilsarkoms
Greek	Fenretinide	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Fenretinid	Lágy szöveti sarcoma kezelése
Italian	Fenretinide	Trattamento dei sarcomi dei tessuti molli
Latvian	Fenretinīds	Mīksto audu sarkomas ārstēšana
Lithuanian	Fenretinidas	Minkštųjų audinių sarkomos gydymas
Polish	Fenretynid	Leczenie mięsaków tkanek miękkich
Portuguese	Fenretinida	Tratamento do sarcoma dos tecidos moles
Romanian	Fenretinidă	Tratamentul sarcomului țesuturilor moi
Slovak	Fenretinid	Liečba sarkómu mäkkých tkanív
Slovenian	Fenretinid	Zdravljenje sarkoma mehkih tkiv
Spanish	Fenretinida	Tratamiento del sarcoma de tejidos blandos
Swedish	Fenretinid	Behandling av mjukdelssarkom
Norwegian	Fenretinid	Behandling av bløtvevssarkom
Icelandic	Fenretíníð	Meðferð við mjúkvefjasarkmeini

¹ At the time of designation