



COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

**PUBLIC SUMMARY OF
POSITIVE OPINION FOR ORPHAN DESIGNATION
OF
ecteinascidin 743
for the treatment of soft tissue sarcoma**

On 30 May 2001, orphan designation (EU/3/01/039) was granted by the European Commission to Pharma Mar SA, Spain, for ecteinascidin 743 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, which tend to behave differently. The cause of soft tissue sarcoma is largely unknown. Soft tissue sarcoma is a serious condition, potentially debilitating and life-threatening.

What are the methods of treatment 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) is usually used as well as surgery. Once the tumour has progressed beyond possible surgery, chemotherapy (using drugs to kill cancer cells) remains the treatment of choice. Several products for treatment of soft tissue sarcoma were authorised for the condition in the Community at the time of submission of the application for orphan drug designation.

Ecteinascidin 743 might be of potential significant benefit for the treatment of soft tissue sarcoma, because it may offer a new way of killing cancer cells. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, soft tissue sarcoma was considered to affect about 23,000 in the European Union.

How is this medicinal product expected to act?

Ecteinascidin 743 is a substance isolated from a small marine animal called sea squirt living on mangrove roots in the Caribbean Sea. Ecteinascidin 743 could exert antitumour activity via several mechanisms, such as bending DNA (the fundamental genetic material) in the cells and thereby making it difficult for the cell to multiply. Furthermore, ecteinascidin 743 might induce a decrease in the speed of cell cycle multiplication and eventually a cell cycle arrest, thereby preventing cancer cells to grow or at least slowing down their growth.

* Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

What is the stage of development of this medicinal product?

The effects of ecteinascidin 743 were evaluated in experimental models. At the time of submission of the application for orphan designation, three clinical trials in patients with soft tissue sarcoma were ongoing and one was completed.

Ecteinascidin 743 was not marketed anywhere worldwide for soft tissue sarcoma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 April 2001 a positive opinion recommending the grant of the above-mentioned designation.

Update: ecteinascidin 743 (Yondelis) is authorised in the European Union as of 17 September 2007 for the treatment of patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on liposarcoma and leiomyosarcoma patients.

For more information please see www.emea.europa.eu

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

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Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Ecteinascidin 743	Treatment of soft tissue sarcoma
Bulgarian	Ектейнасцидин	Лечение на сарком на меките тъкани
Czech	Ecteinascidin 743	Léčba sarkomu měkkých tkání
Danish	Ecteinascidin 743	Behandling af bløddelssarkom
Dutch	Ecteinascidin 743	Behandeling weke delen sarcoom
Estonian	Ecteinascidin 743	Pehmele kudede sarkoomi ravi
Finnish	Ecteinascidin 743	Pehmytkudossarkooman hoito
French	Ecteinascidine 743	Traitement des sarcomes des tissus mous
German	Ecteinascidin 743	Behandlung des Weichteilsarkoms
Greek	Ecteinascidin 743	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Ecteinascidin 743	Lágy szöveti sarcoma kezelése
Italian	Ecteinascidin 743	Trattamento dei sarcomi dei tessuti molli
Latvian	Ekteinascidīns 743	Mīksto audu sarkomas ārstēšana
Lithuanian	Ekteinascidinas 743	Minkštųjų audinių sarkomos gydymas
Maltese	Ecteinascidin 743	Kura tas-sarkoma tat-tessuti rotob
Polish	Ekteinascydyna 743	Leczenie mięsaków tkanek miękkich
Portuguese	Ecteinascidin 743	Tratamento do sarcoma dos tecidos moles
Romanian	Ecteinascidin 743	Tratamentul sarcomului țesuturilor moi
Slovak	Ekteinascidín 743	Liečba sarkómu mäkkých tkanív
Slovenian	Ekteinascidin 743	Zdravljenje sarkoma mehkih tkiv
Spanish	Ecteinascidina 743	Tratamiento del sarcoma de tejidos blandos
Swedish	Ecteinascidin 743	Behandling av mjukdelssarkom