



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of doxorubicin hydrochloride (liposomal) for the treatment of soft tissue sarcoma

On 27 October 2006, orphan designation (EU/3/06/410) was granted by the European Commission to GP-Pharm S.A., Spain, for doxorubicin hydrochloride (liposomal) 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 approximately 2.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 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 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 tissue sarcoma were authorised for the condition in some countries 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 doxorubicin hydrochloride (liposomal) 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?

Doxorubicin is a drug to treat tumours. Doxorubicin interferes with the production of genetic material and can kill rapidly dividing cells such as tumour cells. In the designated product, doxorubicin is located in the wall of a liposome (a microscopic particle made out of the same material as a cell

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

membrane that can be filled with drugs). This allows the liposomes to circulate for prolonged periods in the blood stream and thus might maintain a longer effect.

What is the stage of development of this medicine?

The effects of doxorubicin hydrochloride (liposomal) 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.

Doxorubicin hydrochloride (liposomal) was not authorised 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 6 September 2006 a positive opinion recommending the grant of the above-mentioned 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 Community) 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.

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**Translations of the active ingredient and indication in all official EU languages,
Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Doxorubicin hydrochloride (liposomal)	Treatment of soft tissue sarcoma
Czech	Doxorubicin hydrochlorid (liposomální)	Léčba sarkomu měkkých tkání
Danish	Doxorubicin hydrochlorid (liposomal)	Behandling af bløddelssarkom
Dutch	Doxorubicinehydrochloride (liposomaal)	Behandeling weke delen sarcoom
Estonian	Doksorubitsiinvesinikkloriid (liposomaalne)	Pehmele kudede sarkoomi ravi
Finnish	Doksorubisiinihydroklorid (liposomeihin) i	Pehmytkudossarkooman hoito
French	Chlorhydrate de doxorubicine (liposomal)	Traitement des sarcomes des tissus mous
German	Doxorubicinhydrochlorid (eingeschlossen in Liposomen)	Behandlung des Weichteilsarkoms
Greek	Υδροχλωρική δοξορουβικίνη (Λιποσωμική)	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Doxorubicin-hidroklorid (liposzómába zárva)	Lágy szöveti sarcoma kezelése
Italian	Doxorubicina cloridrato (liposomale)	Trattamento dei sarcomi dei tessuti molli
Latvian	Doksorubicīna hidrohlorīds (īposomu)	Mīksto audu sarkomas ārstēšana
Lithuanian	Doksorubicino hidrochloridas (liposominis)	Minkštųjų audinių sarkomos gydymas
Polish	Doksorubicyny chlorowodorek (w liposomach)	Leczenie mięsaków tkanek miękkich
Portuguese	Cloridrato de doxorubicina (lipossómico)	Tratamento do sarcoma dos tecidos moles
Slovak	Doxorubicíniumchlorid (lipozomálny)	Liečba sarkómu mäkkých tkanív
Slovenian	Doksorubicin hidroklorid (liposomni)	Zdravljenje sarkoma mehkih tkiv
Spanish	Doxorubicina clorhidrato (liposomal)	Tratamiento del sarcoma de tejidos blandos
Swedish	doxorubicinhydroklorid (liposomal)	Behandling av mjukdelssarkom
Norwegian	doksorubicinhydroklorid (liposomalt)	Behandling av bløtvevssarkom
Icelandic	Doxórúbicín hýdróklóríð (í lípósómunum)	Meðferð við mjúkvefjasarkmeini