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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Doxorubicin(6-maleimidocaproyl)hydrazone for the treatment of soft tissue sarcoma

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 26 March 2014, orphan designation (EU/3/14/1258) was granted by the European Commission to Eudax Srl, Italy, for doxorubicin(6-maleimidocaproyl)hydrazone for the treatment of soft tissue sarcoma.

The sponsorship was transferred to Pharma Gateway AB, Sweden, in March 2015.

What is soft tissue sarcoma?

Soft tissue sarcoma is a type of cancer that affects the soft, supportive tissues of the body. It can occur in muscles, blood vessels, fat tissue or in other tissues that support, surround and protect organs. Patients with soft tissue sarcoma do not usually have symptoms in the early stages of the disease. First symptoms appear when the tumour grows large enough to cause swelling and pain.

Soft tissue sarcoma is a long-term debilitating and life-threatening disease, particularly when the cancer has spread to other parts of the body.

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

At the time of designation, soft tissue sarcoma affected not more than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 103,000 people*, and is below

*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.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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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, the main treatment for early-stage soft tissue sarcoma was surgery. For large sarcomas, surgery was usually followed by radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer) to kill any cancerous cells that were left behind. Several medicines were authorised in the EU for the treatment of soft tissue sarcoma.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with soft tissue sarcoma because early studies in patients showed better response rates than existing treatments. These assumptions 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?

Doxorubicin has been available as an anticancer medicine since the 1960s. It is a cytotoxic (cell-killing) medicine that belongs to the group 'anthracyclines'. It works by interfering with the DNA within cells, preventing them from making more copies of DNA and making proteins. This means that cancer cells cannot divide and eventually die.

In this medicine, doxorubicin is linked to a small molecule that attaches to albumin, a blood protein which is known to accumulate in tumours such as soft tissue sarcoma. Albumin is therefore expected to act as a carrier and transport the medicine through the blood vessels directly to the tumour environment where doxorubicin is expected to be released. Once released, doxorubicin is expected to act to kill the cancer cells, thus improving the symptoms or the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with soft tissue sarcoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for soft tissue sarcoma. Orphan designation of the medicine had been granted in the United States for soft tissue sarcoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2014 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Doxorubicin(6-maleimidocaproyl)hydrazone	Treatment of soft tissue sarcoma
Bulgarian	Доксорубицин (6-малеимидокапроил) хидразон	Лечение на сарком на меките тъкани
Czech	Doxorubicin(6-maleimidocaproyl) hydrazón	Léčba sarkomu měkkých tkání
Croatian	Doksorubicin(6-maleimidokaproil)hidrazon	Liječenje sarkoma mekih tkiva
Danish	Doxorubicin(6-maleimidocaproyl)hydrazon	Behandling af bløddelssarkom
Dutch	Doxorubicine(6-maleimidocaproyl)hydrazone	Behandeling weke delen sarcoom
Estonian	Doksorubitsiin(6-maleimidokaproüül)hüdrasoon	Pehmete kudede sarkoomi ravi
Finnish	Doksorubisiini(6-maleimidokaproyyli)hydratsoni	Pehmytkudossarkooman hoito
French	(6-maléimidocaproyl)hydrazone doxorubicine	Traitement des sarcomes des tissus mous
German	Doxorubicin (6-Maleimidocaproyl)hydrazon	Behandlung des Weichteilsarkoms
Greek	Δοξορουβικίνη(6-μαλεϊμιδο καπροϋλ)υδραζόνη	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Doxorubicin(6-maleimidokaproil)hidrazon	Lágy szöveti sarcoma kezelése
Italian	Doxorubicina(6-maleimidocaproil)idrazone	Trattamento dei sarcomi dei tessuti molli
Latvian	Doksorubicīn(6-maleimidokaproil)hidrazons	Mīksto audu sarkomas ārstēšana
Lithuanian	Doksorubicin(6-maleimidokaproil)hidrazonas	Minkštųjų audinių sarkomos gydymas
Maltese	Doxorubicin(6-maleimidocaproyl)hydrazone	Kura tas-sarkoma tat-tessuti rotob
Polish	Hydrazon 6-maleimidokaproilodoksorubicyny	Leczenie mięsaków tkanek miękkich
Portuguese	Doxorrubicina (6-maleimidocaproil)hidrazona	Tratamento do sarcoma dos tecidos moles
Romanian	Doxorubicină(6-maleimidocaproil)hidrazonă	Tratamentul sarcomului ţesuturilor moi
Slovak	Doxorubicín (6-maleimidokaproyl) hydrazón	Liečba sarkómu mäkkých tkanív
Slovenian	Doksorubicin(6-maleimidokaproil)hidrazon	Zdravljenje sarkoma mehkih tkiv
Spanish	Doxorubicina (6-maleimidocaproil) hidrazona	Tratamiento del sarcoma de tejidos blandos
Swedish	Doxorubicin(6-maleimidokaproyl)hydrazon	Behandling av mjukdelssarkom
Norwegian	Doksorubicin(6-maleimidokaproyl)hydrazon	Behandling av bløtvevssarkom
Icelandic	Doxórúbisín(6-maleímíðókapróýl)hýdrasón	Meðferð við mjúkvefjasarkmeini

[1] At the time of designation