



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

7-Ethyl-10-hydroxy-camptothecin for the treatment of soft tissue sarcoma

On 25 July 2019, orphan designation EU/3/19/2181 was granted by the European Commission to Cebiotex S.L., Spain, for 7-ethyl-10-hydroxy-camptothecin for the treatment of soft tissue sarcoma.

What is soft tissue sarcoma?

Soft tissue sarcoma is a 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 cancer 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 approximately 3.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 187,000 people*, and is below 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 treatments for soft tissue sarcoma were surgery and chemotherapy (medicines to treat cancer). Radiotherapy (treatment with radiation) was also used. Several medicines have been 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. Laboratory studies show that the medicine, given in combination with doxorubicin, a standard chemotherapy treatment for the condition, is more active against cancer cells than doxorubicin alone. In addition, the sponsor provided data from the literature

*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. This represents a population of 518,400,000 (Eurostat 2019).



showing that the medicine may be added to chemotherapy or radiotherapy. This assumption 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?

7-Ethyl-10-hydroxy-camptothecin is the active form of a well-known anti-cancer medicine called irinotecan. It belongs to the group 'topoisomerase inhibitors' and blocks an enzyme called topoisomerase I, which is involved in the division of DNA. When the enzyme is blocked, the DNA strands break. This prevents the cancer cells from dividing and they eventually die. The active substance is incorporated into a membrane that will be implanted into the patient's tissue after they have received surgery. The membrane will allow the slow release of the cancer medicine into the remaining tissue where it will control and kill cancer cells.

What is the stage of development of this medicine?

The effects of 7-ethyl-10-hydroxy-camptothecin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with 7-ethyl-10-hydroxy-camptothecin in patients with soft tissue sarcoma were ongoing.

At the time of submission, 7-ethyl-10-hydroxy-camptothecin was not authorised anywhere in the EU for the treatment of soft tissue sarcoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 June 2019, 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:

Contact details of the current sponsor for this orphan designation can be found on [EMA website](#).

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	7-ethyl-10-hydroxycamptothecin	Treatment of soft tissue sarcoma
Bulgarian	7-етил-10-хидроксикампототецин	Лечение на сарком на меките тъкани
Croatian	7-etil-10-hidroksikamptotekin	Liječenje sarkoma mekih tkiva
Czech	7-ethyl-10-hydroxycamptothecine	Léčba sarkomu měkkých tkání
Danish	7-ethyl-10-hydroxycamptothecin	Behandling af bløddelsarkom
Dutch	7-ethyl-10-hydroxycamptothecine	Behandeling weke delen sarcoom
Estonian	7-etüül-10-hüdroksükamptotetsiin	Pehmeete kudede sarkoomi ravi
Finnish	7-etyyli-10-hydroksikamptotesiini	Pehmytkudossarkooman hoito
French	7-éthyl-10-hydroxycamptothécine	Traitement des sarcomes des tissus mous
German	7-Ethyl-10-hydroxycamptothecin	Behandlung des Weichteilsarkoms
Greek	7-αιθυλο-10-υδροξυ-καμπτοθεκίνη	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	7-etil-10-hidroxikamptotecin	Lágy szöveti sarcoma kezelése
Italian	7-etil-10-idrossicamptotecina	Trattamento dei sarcomi dei tessuti molli
Latvian	7-etil-10-hidroksikamptotecīns	Mīksto audu sarkomas ārstēšana
Lithuanian	7-etil-10-hidroksikamptotecinas	Minkštųjų audinių sarkomos gydymas
Maltese	7-etil-10-idrossikamptotecina	Kura tas-sarkoma tat-tessuti rotob
Polish	7-etylo-10-hydroksykamptotecyna	Leczenie mięsaków tkanek miękkich
Portuguese	7-etil-10-hidroxicamptotecina	Tratamento do sarcoma dos tecidos moles
Romanian	7-etil-10-hidroxicamptotecină	Tratamentul sarcomului țesuturilor moi
Slovak	7-etyl-10-hydroxykamptotecín	Liečba sarkómu mäkkých tkanív
Slovenian	7-etil-10-hidroksikamptotekin	Zdravljenje sarkoma mehkih tkiv
Spanish	7-etil-10-hidroxicamptotecina	Tratamiento del sarcoma de tejidos blandos
Swedish	7-etyl-10-hydroxy-kamptotecin	Behandling av mjukdelssarkom
Norwegian	7-etyl-10-hydroksykamptotecin	Behandling av bløtvevssarkom
Icelandic	7-etyl-10-hýdroxýkamptótécín	Meðferð við mjúkvefjasarkmeini

¹ At the time of designation