



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Camsirubicin for the treatment of soft tissue sarcoma

On 13 November 2019, orphan designation EU/3/19/2221 was granted by the European Commission to Monopar Therapeutics S.A.R.L, France, for camsirubicin for the treatment of soft tissue sarcoma.

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 approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 181,000 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 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 cancer cells that were left behind. Several medicines were authorised in the EU for the treatment of soft tissue sarcoma, including doxorubicin.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with soft tissue sarcoma because evidence from studies in patients suggest it can

*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).



be given for longer without damaging the heart than a similar authorised treatment, doxorubicin, and this may improve response.

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?

The medicine is similar to an existing cancer medicine, doxorubicin, which has been shown to be effective in treating soft-tissue sarcoma. It works by interfering with the DNA within cells, preventing them from making more copies of DNA and making proteins. This means that the sarcoma cells cannot divide and eventually die. In contrast to doxorubicin, which can only be given in limited doses because it also damages heart muscle, camsirubicin has a slightly different structure and its effects on the heart are expected to be less damaging.

What is the stage of development of this medicine?

The effects of camsirubicin have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 10 October 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	Camsirubicin	Treatment of soft tissue sarcoma
Bulgarian	Камсирубицин	Лечение на сарком на меките тъкани
Croatian	Kamsirubicin	Liječenje sarkoma mekih tkiva
Czech	Camsirubicin	Léčba sarkomu měkkých tkání
Danish	Camsirubicin	Behandling af bløddelssarkom
Dutch	Camsirubicine	Behandeling weke delen sarcoom
Estonian	Kamsirubitsiin	Pehmele kudede sarkoomi ravi
Finnish	Kamsirubisiini	Pehmytkudossarkooman hoito
French	Camsirubicine	Traitement des sarcomes des tissus mous
German	Camsirubicin	Behandlung des Weichteilsarkoms
Greek	Καμσιρουμπικίνη	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Camsirubicin	Lágy szöveti sarcoma kezelése
Italian	Camsirubicina	Trattamento dei sarcomi dei tessuti molli
Latvian	Kamsirubicīns	Mīksto audu sarkomas ārstēšana
Lithuanian	Kamsirubicinas	Minkštųjų audinių sarkomos gydymas
Maltese	Kamsirubiċin	Kura tas-sarkoma tat-tessuti rotob
Polish	Kamsirubicyna	Leczenie mięsaków tkanek miękkich
Portuguese	Camsirubicina	Tratamento do sarcoma dos tecidos moles
Romanian	Camsirubicin	Tratamentul sarcomului țesuturilor moi
Slovak	Camsirubicin	Liečba sarkómu mäkkých tkanív
Slovenian	Kamsirubicin	Zdravljenje sarkoma mehkih tkiv
Spanish	Camsirubicina	Tratamiento del sarcoma de tejidos blandos
Swedish	Camsirubicin	Behandling av mjukdelssarkom
Norwegian	Kamsirubicin	Behandling av bløtvevssarkom
Icelandic	Camsirubicin	Meðferð við mjúkvefjasarkmeini

¹ At the time of designation