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## Public summary of opinion on orphan designation

### Vinorelbine tartrate for the treatment of soft tissue sarcoma

On 11 January 2019, orphan designation (EU/3/18/2133) was granted by the European Commission to TLC Biopharmaceuticals B.V., the Netherlands, for vinorelbine tartrate 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 155,000 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 vinorelbine tartrate might be of significant benefit for patients with soft tissue sarcoma. Laboratory studies show that the medicine is more active against cancer cells than doxorubicin, a standard chemotherapy treatment for the condition. In

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\*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 517,400,000 (Eurostat 2018).

addition, early studies in patients showed that vinorelbine tartrate used together with the medicine cyclophosphamide may improve patients' survival. 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?**

Vinorelbine tartrate belongs to a class of cancer medicines known as vinca alkaloids. It attaches to a protein in cells called tubulin, which is important in the formation of the internal 'skeleton' that cells need to assemble when they divide and multiply. By attaching to tubulin in cancer cells, vinorelbine tartrate stops the formation of this skeleton, preventing the division and growth of the cancer cells.

Vinorelbine tartrate is already authorised in the EU for the treatment of breast cancer and non-small cell lung cancer. In this medicine, vinorelbine tartrate is contained in fatty particles called liposomes which protect it from being broken down and allow it to be delivered into cancer cells.

## **What is the stage of development of this medicine?**

The effects of vinorelbine tartrate 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, vinorelbine tartrate was not authorised anywhere in the EU for soft tissue sarcoma. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 December 2018 recommending the granting of this designation.

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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 [the 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 orphan condition in all official EU languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Vinorelbine tartrate	Treatment of soft tissue sarcoma
Bulgarian	Винорелбин тартарат	Лечение на сарком на меките тъкани
Croatian	Vinorelbin tartrat	Liječenje sarkoma mekih tkiva
Czech	Vinorelbin tartrát	Léčba sarkomu měkkých tkání
Danish	Vinorelbintartrat	Behandling af bløddelssarkom
Dutch	Vinorelbintartraat	Behandeling weke delen sarcoom
Estonian	Vinorelbiintartraat	Pehmele kudede sarkoomi ravi
Finnish	Vinorelbiinitartraatti	Pehmytkudossarkooman hoito
French	Tartrate de vinorelbine	Traitement des sarcomes des tissus mous
German	Vinorelbin-Tartrat	Behandlung des Weichteilsarkoms
Greek	Τρυγική βινoreλβίνη	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Vinorelbin tartarát	Lágy szöveti sarcoma kezelése
Italian	Vinorelbina tartrato	Trattamento dei sarcomi dei tessuti molli
Latvian	Vinorelbīna tartrāts	Mīksto audu sarkomas ārstēšana
Lithuanian	Vinorelbino tartratas	Minkštųjų audinių sarkomos gydymas
Maltese	Vinorelbina tartrate	Kura tas-sarkoma tat-tessuti rotob
Polish	Winian winorelbiny	Leczenie mięsaków tkanek miękkich
Portuguese	Tartarato de vinorelbina	Tratamento do sarcoma dos tecidos moles
Romanian	Tartrat de vinorelbina	Tratamentul sarcomului țesuturilor moi
Slovak	Vinorelbín vínán	Liečba sarkómu mäkkých tkanív
Slovenian	Vinorelbin tartrat	Zdravljenje sarkoma mehkih tkiv
Spanish	Tartrato de vinorelbina	Tratamiento del sarcoma de tejidos blandos
Swedish	Vinorelbintartrat	Behandling av mjukdelssarkom
Norwegian	Vinorelbintartrat	Behandling av bløtvevssarkom
Icelandic	Vinorelbíntartrat	Meðferð við mjúkvefjasarkmeini

<sup>1</sup> At the time of designation