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Public summary of opinion on orphan designation

Paclitaxel for the treatment of soft tissue sarcoma

On 17 October 2019, orphan designation EU/3/19/2215 was granted by the European Commission to Boyd Consultants Limited, Ireland, for paclitaxel 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 4.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 238,000 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. Early data showed improvements in angiosarcoma (sarcoma affecting blood vessels) when patients were treated with paclitaxel, given by mouth, plus another medicine, 4-oxo-4H-chromene-2-carboxylic acid (2-(2-4-(2-(6,7-dimethoxy-3,4-dihydro-1H-isoquinolin-2-yl)-ethyl)-phenyl-2H-tetrazol-5-yl)-4,5-dimethoxy-phenyl)-amide (see orphan

*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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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designation EU/3/19/2207), that allows paclitaxel to work when given by mouth rather than infusion into a vein. Giving paclitaxel this way may reduce its side effects, so the combination could be of benefit in elderly patients who might be unfit for standard chemotherapy. 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?

Paclitaxel is a cancer medicine that has been used in the EU for many years and is normally given by infusion into a vein. Paclitaxel blocks the ability of cancer cells to break down their internal 'skeleton' that allows them to divide and multiply. With their skeleton still in place, the cells cannot divide and they eventually die. The other medicine, 4-oxo-4H-chromene-2-carboxylic acid (2-(2-4-(2-(6,7-dimethoxy-3,4-dihydro-1H-isoquinolin-2-yl)-ethyl)-phenyl-2H-tetrazol-5-yl)-4,5-dimethoxy-phenyl)-amide, blocks the effect of P-glycoprotein, a protein in the body that helps remove foreign substances from cells. The combination is expected to allow paclitaxel to work when given by mouth, helping it pass through cells in the gut and then into cancer cells. This is expected to allow paclitaxel by mouth to slow down the growth of sarcomas and reduce side effects that may be caused by giving paclitaxel by infusion into a vein.

What is the stage of development of this medicine?

The effects of paclitaxel have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 12 September 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Paclitaxel	Treatment of soft tissue sarcoma
Bulgarian	Паклитаксел	Лечение на сарком на меките тъкани
Croatian	Paclitaksel	Liječenje sarkoma mekih tkiva
Czech	Paclitaxel	Léčba sarkomu měkkých tkání
Danish	Paclitaxel	Behandling af bløddelssarkom
Dutch	Paclitaxel	Behandeling weke delen sarcoom
Estonian	Paklitakseel	Pehmete kudede sarkoomi ravi
Finnish	Paklitakseli	Pehmytkudossarkooman hoito
French	Paclitaxel	Traitement des sarcomes des tissus mous
German	Paclitaxel	Behandlung des Weichteilsarkoms
Greek	Πακλιταξέλη	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Paclitaxel	Lágy szöveti sarcoma kezelése
Italian	Paclitaxel	Trattamento dei sarcomi dei tessuti molli
Latvian	Paklitaksels	Mīksto audu sarkomas ārstēšana
Lithuanian	Paklitakselis	Minkštųjų audinių sarkomos gydymas
Maltese	Paclitaxel	Kura tas-sarkoma tat-tessuti rotob
Polish	Paklitaksel	Leczenie mięsaków tkanek miękkich
Portuguese	Paclitaxel	Tratamento do sarcoma dos tecidos moles
Romanian	Paclitaxel	Tratamentul sarcomului ţesuturilor moi
Slovak	Paklitaxel	Liečba sarkómu mäkkých tkanív
Slovenian	Paklitaksel	Zdravljenje sarkoma mehkih tkiv
Spanish	Paclitaxel	Tratamiento del sarcoma de tejidos blandos
Swedish	Paklitaxel	Behandling av mjukdelssarkom
Norwegian	Paklitaksel	Behandling av bløtvevssarkom
Icelandic	Paklítaxel	Meðferð við mjúkvefjasarkmeini

[1] At the time of designation