



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Lipid-complexed cisplatin for the treatment of osteosarcoma

On 5 August 2013, orphan designation (EU/3/13/1169) was granted by the European Commission to Richardson Associates Regulatory Affairs Ltd, United Kingdom, for lipid-complexed cisplatin for the treatment of osteosarcoma.

#### What is osteosarcoma?

Osteosarcoma is a type of cancer that affects the bone-forming cells. It usually affects the growing bone and occurs mainly in teenagers and young adults. Although it can develop in any bone in the body, it most often starts in the bones around the knee joint in the area of bone with the fastest growth. The commonest symptom is pain, which may be worse at night. Symptoms also depend on where the cancer has spread to. The most common site of spread for this cancer is the lungs.

Osteosarcoma is a long-term debilitating and life-threatening disease that may require patients to undergo limb amputations and is associated with poor long-term survival.

#### What is the estimated number of patients?

At the time of designation, osteosarcoma affected less than 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 127,000 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 osteosarcoma were surgery and chemotherapy (medicines to treat cancer). The goal of surgery was to remove cancerous bone while preserving as much skeletal function as possible. Chemotherapy was normally used to shrink the tumour before surgery and also after surgery to stop any remaining cancer cells from spreading. A number of chemotherapy medicines, including cisplatin, were authorised in the EU for this condition.

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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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).



The sponsor has provided sufficient information to show that lipid-complexed cisplatin might be of significant benefit to patients with osteosarcoma because early studies show that it might be effective in treating osteosarcoma that has spread to the lungs and has come back after treatment with standard medicines. 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?**

Lipid-complexed cisplatin is a new formulation of cisplatin, an anticancer medicine that has been used for osteosarcoma for several years. Cisplatin belongs to a class of medicines called 'alkylating agents' and works by attaching to the DNA of cells while they are reproducing, which stops cell division. As a result, cancer cells cannot divide and eventually die, slowing down the growth of tumours.

In this medicine, cisplatin is attached to lipid (fat) molecules so that the medicine can be inhaled into the lungs where cisplatin is then released gradually over time to act against cancer cells that have spread to the lungs. The fat molecules attached to cisplatin are expected to keep the cisplatin in the patient's lungs for longer than 'free' cisplatin and thereby allow it to act over a longer period of time.

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

The effects of lipid-complexed cisplatin 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 osteosarcoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for osteosarcoma. Orphan designation had been granted in the United States for osteosarcoma that had spread to the lungs.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2013 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:

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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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Lipid-complexed cisplatin	Treatment of osteosarcoma
Bulgarian	цисплатин в липиден комплекс	Лечение на остеосарком
Croatian	Kompleks cisplatina s lipidima	Liječenje Osteosarkom
Czech	Lipidový komplex cisplatiny	Léčba osteosarkomu
Danish	Lipid-cisplatin kompleks	Behandling af osteosarcoma
Dutch	Lipide-gecomplexeerd cisplatine	Behandeling van osteosarcoma
Estonian	Lipiidkompleksiga tsisplatiin	Osteosarkoomi ravi
Finnish	Lipideihin yhdistetty sisplatiini	Osteosarkoman hoito
French	Complexe Cisplatine-Lipides	Traitement des ostéosarcomes
German	Lipid-komplexiertes Cisplatin	Behandlung von Osteosarkomen
Greek	Σισπλατίνη σε σύμπλεγμα λιπιδίων	Θεραπεία του οστεοσαρκώματος
Hungarian	Cisplatin lipid komplex.	Osteosarcoma kezelése
Italian	Cisplatino complessato con lipidi	Trattamento dell'osteosarcoma
Latvian	Lipīdu kompleksa cisplatīns	Osteosarkomas ārstēšana
Lithuanian	Lipidų-komplekso cisplatina	Osteosarkomos gydymas
Maltese	Cisplatin magħqud ma' lipidi	Kura ta' l-osteosarkoma
Polish	Kompleks lipidowy cisplatyny	Leczenie mięsaka kościopochodnego
Portuguese	Complexo lipídico de Cisplatina	Tratamento do osteosarcoma
Romanian	Cisplatină în complex lipidic	Tratamentul osteosarcomului
Slovak	Lipidová cisplatina	Liečba osteosarkómu
Slovenian	Lipidni kompleks s cisplatinom	Zdravljenje osteosarkoma
Spanish	Complejo lipídico de cisplatino	Tratamiento del osteosarcoma
Swedish	Lipidbundet cisplatin	Behandling av osteosarkom
Norwegian	Lipidbundet cisplatin	Behandling av osteosarkom
Icelandic	Fitusamsett cisplatin	Meðferð við beinsarkmeini

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