



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cisplatin for the treatment of malignant mesothelioma

On 29 August 2016, orphan designation (EU/3/16/1719) was granted by the European Commission to PlumeStars s.r.l., Italy, for cisplatin for the treatment of malignant mesothelioma.

What is malignant mesothelioma?

Malignant mesothelioma is a cancer that affects the mesothelial cells (found on the inner linings of the organs), mainly in the pleura (the lining of the lungs) and in the peritoneum (the lining of the abdominal cavity). It is usually caused by exposure to asbestos. Mesothelioma of the pleura causes difficulty breathing and chest pain, and mesothelioma of the peritoneum causes ascites (a build-up of fluid in the abdomen) and abdominal pain.

Malignant mesothelioma is life-threatening because it may lead to bowel obstruction, heart or breathing problems and lung infections. Patients have very poor survival, only living for a year, on average, after diagnosis.

What is the estimated number of patients affected by the condition?

At the time of designation, malignant mesothelioma affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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 malignant mesothelioma were surgery and chemotherapy (medicines to treat cancer) with or without radiotherapy (treatment with radiation). If the disease was too advanced for surgery, chemotherapy alone was used. One medicine, pemetrexed, was authorised throughout the EU for the treatment of malignant pleural mesothelioma. A second medicine, raltitrexed, was authorised for the treatment of malignant pleural mesothelioma in some European countries.

*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 513,700,000 (Eurostat 2016).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with malignant mesothelioma because early studies in experimental models showed that the medicine reduced any tumours left in the lungs after surgery. 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?

Cisplatin is a cancer medicine that has been used to treat various types of cancer for several years. It belongs to a class of medicines called 'alkylating agents' and works by attaching to the DNA of cells, preventing them from multiplying. As a result, cancer cells eventually die, slowing down the growth of tumours.

This medicine consists of a thin and flexible film of material loaded with cisplatin, which is to be implanted in the affected lung during surgery to prevent tumour growth. The film releases cisplatin gradually over time to act against cancer cells that may not have been removed during surgery.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with malignant mesothelioma had been started.

At the time of submission, cisplatin was not authorised anywhere in the EU for malignant mesothelioma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2016 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, on the medicine's [rare disease designations page](#).

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	Cisplatin	Treatment of malignant mesothelioma
Bulgarian	Цисплатин	Лечение на малигнен мезотелиом
Croatian	Cisplatin	Liječenje malignog mezotelioma
Czech	Cisplatina	Léčba maligního mezoteliomu
Danish	Cisplatin	Behandling af malignt mesotheliom
Dutch	Cisplatin	Behandeling van maligne mesothelioom
Estonian	Cisplatin	Pahaloomulise mesotelioomi ravi
Finnish	Sisplatiini	Malignin mesoteliooman hoito
French	Cisplatine	Traitement du mésothéliome malin
German	Cisplatin	Behandlung des malignen Mesothelioms
Greek	Σισπλατίνη	Θεραπεία κακοήθους μεσοθηλιώματος
Hungarian	Cisplatin	Roszzindulatú mesothelioma kezelése
Italian	Cisplatino	Trattamento del mesotelioma maligno
Latvian	Cisplatīns	Ļaundabīgas mezoteliomas ārstēšana
Lithuanian	Cisplatina	Piktybinės mezoteliomos gydymas
Maltese	Cisplatin	Kura tal-mesoteljoma malinna
Polish	Cisplatyna	Leczenie złośliwego międzybłoniaka
Portuguese	Cisplatina	Tratamento do Mesotelioma maligno
Romanian	Cisplatină	Tratamentul mezoteliomului malign
Slovak	Cisplatina	Liečba malígneho mezoteliómu
Slovenian	Cisplatin	Zdravljenje malignega mezotelioma
Spanish	Cisplatino	Tratamiento del mesotelioma maligno
Swedish	Cisplatin	Behandling av malignt mesoteliom
Norwegian	Cisplatin	Behandling av malignt mesoteliom
Icelandic	Cisplatin	Meðferð við illkynja miðþekjuæxli

¹ At the time of designation