13 July 2016 **CONFIDENTIAL**
EMA/COMP/399655/2016
EMA/OD/101/16
Committee for Orphan Medicinal Products

Opinion of the Committee for Orphan Medicinal Products on orphan medicinal product designation

Medicinal product

Active ingredient: Cisplatin

Sponsor

Name or corporate name of sponsor: PlumeStars s.r.l.

Permanent address of sponsor: Strada Inzani 1
43125 Parma
Italy

Indication

Orphan indication: Treatment of malignant mesothelioma

Basis for opinion

Pursuant to Article 5 of Regulation (EC) No 141/2000 of 16 December 1999, PlumeStars s.r.l. submitted to the European Medicines Agency on 23 May 2016 an application for orphan medicinal product designation for the above-mentioned medicinal product.

The procedure started on 13 June 2016.

Opinion

1. The COMP, having considered the application in accordance with Article 5 of Regulation (EC) No 141/2000 of 16 December 1999, is of the opinion that:

- the medicinal product satisfies the criteria for designation as laid out in the first paragraph of Article 3(1)(a), Regulation (EC) No 141/2000 of 16 December 1999; and
- the sponsor has established, as required under Article 3(1)(b), Regulation (EC) No 141/2000 of 16 December 1999, that although a satisfactory method of treatment of the condition in question has been authorised in the European Union, the above-mentioned medicinal product will be of significant benefit to those affected by that condition.

The COMP, therefore, recommends the granting of orphan medicinal product designation for the above-mentioned medicinal product in respect of the above-mentioned indication.

2. The grounds for the opinion on orphan medicinal product designation are set out in the annex.

This opinion is forwarded to the European Commission and to the sponsor, together with its annex.

Annex

Grounds for the opinion on orphan medicinal product designation

The sponsor PlumeStars s.r.l. submitted on 23 May 2016 an application for designation as an orphan medicinal product to the European Medicines Agency for a medicinal product containing cisplatin for treatment of malignant mesothelioma (hereinafter referred to as “the condition”). The application was submitted on the basis of Article 3(1)(a) first paragraph of Regulation (EC) No 141/2000 on orphan medicinal products.

Having examined the application, the COMP considered that the sponsor has established the following:

- the intention to treat the condition with the medicinal product containing cisplatin was considered justified based on a pre-clinical in vivo model of the condition showing a reduction in tumour nodules;
- the condition is life-threatening due to the invasion of the pleura leading to pleural effusions, dyspnoea and malignant ascites. Local invasion may also result in obstruction of the superior vena cava, cardiac tamponade, and spinal cord compression. Patients with pleural mesothelioma usually die due to increasing tumour bulk that gradually fills the hemithorax causing progressive respiratory compromise (“incarceration” of the lungs), pneumonia, or myocardial dysfunction with arrhythmias. In patients with peritoneal mesothelioma, distension due to ascites, abdominal pain, and organ impairment such as bowel obstruction are observed;
- the condition was estimated to be affecting less than 1 in 10,000 persons in the European Union, at the time the application was made.

Thus, the requirements under Article 3(1)(a) of Regulation (EC) No 141/2000 on orphan medicinal products are fulfilled.

In addition, although satisfactory methods of treatment of the condition have been authorised in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing cisplatin will be of significant benefit to those affected by the condition. The sponsor has provided preclinical data that demonstrate a reduction in pleural tumour nodules which may translate into improved management of local residual disease. The Committee considered that this constitutes a clinically relevant advantage.

Thus, the requirement under Article 3(1)(b) of Regulation (EC) No 141/2000 on orphan medicinal products is fulfilled.

The COMP concludes that the requirements laid down in Article (3)(1) (a) and (b) of Regulation (EC) No 141/2000 on orphan medicinal products are fulfilled. The COMP therefore recommends the designation of this medicinal product, containing cisplatin as an orphan medicinal product for the orphan indication: treatment of malignant mesothelioma.