EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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EMA/COMP/742438/2016
EMA/OD/268/16
Committee for Orphan Medicinal Products

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

Medicinal product

Active ingredient: Thalidomide

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 hereditary haemorrhagic telangiectasia

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 25 October 2016 an application for orphan medicinal product designation for the above-mentioned medicinal product.

The procedure started on 21 November 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 there exists no satisfactory method of treatment of the condition in question that has been authorised in the European Union.

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 25 October 2016 an application for designation as an orphan medicinal product to the European Medicines Agency for a medicinal product containing thalidomide for treatment of hereditary haemorrhagic telangiectasia (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 thalidomide was considered justified based on preliminary clinical data showing reduction of frequency, intensity and duration of nasal epistaxis and improvement of haemoglobin levels in patients affected by the condition;
- the condition is life-threatening and chronically debilitating due to arteriovenous malformations in different organs, leading to recurrent bleeding from the nasal mucosa with development of severe anaemia, and to potentially fatal bleeding in the stomach, gut, brain, liver and lungs;
- the condition was estimated to be affecting not more than 2 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.

The sponsor has also established that there exists no satisfactory method of treatment that has been authorised in the European Union for patients affected by the condition.

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 thalidomide as an orphan medicinal product for the orphan indication: treatment of hereditary haemorrhagic telangiectasia.