



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Recommendation for maintenance of orphan designation at the time of marketing authorisation

Scenesse (afamelanotide) for the treatment of erythropoietic protoporphyria

During its meeting of 11 to 13 November 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/08/541 for Scenesse (afamelanotide)<sup>1</sup> as an orphan medicinal product for the treatment of erythropoietic protoporphyria. The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other methods of treatment. The COMP recommended that the orphan designation of the medicine be maintained<sup>2</sup>.

### Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Scenesse for:

‘prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP)’.

This falls within the scope of the product’s designated orphan indication, which is: ‘treatment of erythropoietic protoporphyria’.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2008. Erythropoietic protoporphyria remains a condition that is debilitating in the long term or life threatening, particularly due to the skin problems, anaemia (low levels of red blood cells) and liver complications.

### Prevalence of the condition

The sponsor provided updated information on the prevalence of erythropoietic protoporphyria based on data from the scientific literature.

<sup>1</sup> Previously known as [Nle4, D-Phe7]-alfa-melanocyte stimulating hormone.

<sup>2</sup> The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with a comparable therapeutic indication cannot be placed on the market.



On the basis of the information provided by the sponsor, the COMP concluded that the prevalence of erythropoietic protoporphyria remains below the ceiling for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was estimated to be less than 0.2 people in 10,000. This is equivalent to fewer than 10,000 people in the EU.

### **Existence of other methods of treatment**

The COMP noted that, at the time of the review of the orphan designation, no treatments were authorised in the EU for patients with erythropoietic protoporphyria.

### **Conclusions**

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Scenesse still meets the criteria for designation as an orphan medicinal product and that it should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Scenesse can be found in the European public assessment report (EPAR) on the Agency's website: [ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports](http://ema.europa.eu/Find%20medicine/Human%20medicines/European%20Public%20Assessment%20Reports).