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Committee for Orphan Medicinal Products

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

Ledaga (chlormethine) for the treatment of cutaneous T-cell lymphoma

During its meeting of 17 to 19 January, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/12/963 for Ledaga (chlormethine) as an orphan medicinal product for the treatment of cutaneous T-cell lymphoma. 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. As other methods of treatment are authorised in the European Union (EU), the COMP also considered whether the medicine is of significant benefit to patients with cutaneous T-cell lymphoma. The COMP recommended that the orphan designation of the medicine be maintained[1].

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

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

'the topical treatment of mycosis fungoides-type cutaneous T-cell lymphoma (MF-type CTCL) in adult patients'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of cutaneous T-cell lymphoma'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2012. Cutaneous T-cell lymphoma remains a debilitating and life-threatening condition because it can develop into a cancer that spreads more quickly and becomes infected, and it may have a large effect on the patient's quality of life, particularly because the skin lesions can cause disfigurement.

[1] 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 the same therapeutic indication cannot be placed on the market.

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Prevalence of the condition

The sponsor provided updated information on the prevalence of cutaneous T-cell lymphoma based on data from the scientific literature.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of cutaneous T-cell lymphoma 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 approximately 2.7 people in 10,000. This is equivalent to a total of around 139,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, other treatments were authorised in the EU for the treatment of cutaneous T-cell lymphoma. They included treatment with ultraviolet light and x-rays as well as cytotoxic medicines (medicines that kill cells that are dividing, such as cancer cells) and interferon alfa (a medicine that helps the immune system to fight against the cancer cells), which were both given by injection.

Significant benefit of Ledaga

The COMP concluded that the claim of a significant benefit of Ledaga in cutaneous T-cell lymphoma is justified on the basis that this gel product offers the possibility for topical (applied to the skin) treatment for patients with this condition. Topical treatment with chlormethine is a therapeutic option for cutaneous T-cell lymphoma in current European guidelines. The COMP considered that the availability of a topical chlormethine product would therefore be a major contribution to patient care.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Ledaga is of significant benefit to patients affected by cutaneous T-cell lymphoma.

Conclusions

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

More information on the COMP assessment can be found in the January 2017 COMP minutes.

Further information on Ledaga 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.