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EMA/COMP/378290/2010
Committee for Orphan Medicinal Products

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

Revolade (eltrombopag olamine) for the treatment of idiopathic thrombocytopenic purpura

During its meeting of 5-6 January 2010, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/07/467 for Revolade (eltrombopag olamine) as an orphan medicinal product for the treatment of idiopathic thrombocytopenic purpura. 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 satisfactory methods of treatment. As other satisfactory methods of treatment for patients with this condition are authorised in the European Union (EU), the COMP also looked at the significant benefit of the product over existing treatments. 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 Revolade for:

'Revolade is indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Revolade may be considered as second-line treatment for adult non-splenectomised patients where surgery is contraindicated.'

This falls within the scope of the product's designated orphan indication, which is: 'treatment of idiopathic thrombocytopenic purpura'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in August 2007. Idiopathic thrombocytopenic purpura remains a condition that is chronically debilitating and life threatening due to the high risk of potentially lethal haemorrhages and post-splenectomy infections.

[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 a comparable therapeutic indication cannot be placed on the market.

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Prevalence of the condition

The sponsor informed the COMP that no changes to the prevalence of the condition had been reported since the orphan designation of Revolade.

On the basis of this information and the knowledge of the COMP, the COMP concluded that the prevalence of idiopathic thrombocytopenic purpura remains below the threshold for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was still estimated to be between 1 and 4 people in 10,000. This is equivalent to a total of between 51,000 and 203,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, several medicines were authorised in the EU for the treatment of idiopathic thrombocytopenic purpura. Intravenous corticosteroids and immunoglobulins were used as first-line treatment in order to increase the platelet counts to a safe range and to minimise the risk of bleeding. Second-line treatment usually involved splenectomy (spleen removal) in order to limit the destruction of the platelets, since the spleen is the most important organ where old platelets are removed from the blood. The orphan medicine Nplate (romiplostim) was also authorised for use in splenectomised patients who did not respond to other treatments, and as second-line treatment for non-splenectomised adults where surgery was contra-indicated. This medicine was available as a powder for solution for injection.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Revolade in the treatment of idiopathic thrombocytopenic purpura is justified on the basis of its potential major contribution to patient care. This is supported by the fact that Revolade will be available as tablets, while the currently authorised products including Nplate are for subcutaneous administration. This is particularly relevant for those patients who have problems with parenteral administration.

The COMP also noted that in clinical studies Revolade was able to increase the platelet counts in the subgroups of patients with idiopathic thrombocytopenic purpura who have had their spleen removed and who did not respond to treatment with medicines such as corticosteroids or immunoglobulins. In addition, secondary analyses showed that the percentage of subjects with any bleeding (Grades 1-4) and clinically significant bleeding (Grades 2-4) was reduced by approximately 50% after the six-month treatment period. The COMP concluded that, because bleeding is a major manifestation of the disease, the increase in platelet count and reduction in the occurrence of bleeding episodes is a valid justification for assuming a clinically relevant advantage of Revolade over existing treatments.

Therefore, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Revolade is of significant benefit for patients affected by idiopathic thrombocytopenic purpura.

Conclusions

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

Further information on the current regulatory status of Revolade 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.