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Committee for Orphan Medicinal Products

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

Mercaptopurine Nova Laboratories (mercaptopurine (oral suspension)) for the treatment of acute lymphoblastic leukaemia

During its meeting of 6-8 September 2011, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/09/628 for Mercaptopurine Nova Laboratories (mercaptopurine (oral suspension)), previously known as Novapurine, as an orphan medicinal product for the treatment of acute lymphoblastic leukaemia. 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 Mercaptopurine Nova Laboratories for:

'the treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and children'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of acute lymphoblastic leukaemia'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2009. Acute lymphoblastic leukaemia remains a condition that is a serious and potentially life-threatening disease, due to the excessive production of abnormal lymphocytes (a type of white blood cell) in the patient's blood.

[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

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of acute lymphoblastic leukaemia 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 still estimated to be approximately 1.2 people in 10,000. This is equivalent to a total of around 61,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, other treatments were authorised in the EU for the treatment of acute lymphoblastic leukaemia. The main treatment was chemotherapy (medicines used to kill cancer cells) followed by or combined with radiotherapy (using radiations to kill cancer cells). Bone marrow transplantation was also used. Mercaptopurine was already available in the EU as 50 mg tablets for the treatment of acute lymphoblastic leukaemia.

Significant benefit over existing treatments

Acute lymphoblastic leukaemia is the most common type of leukaemia in young children. The COMP concluded that the claim of a significant benefit of Mercaptopurine Nova Laboratories in acute lymphoblastic leukaemia is justified because the medicine offers a liquid form of mercaptopurine which may be given to children who have difficulty swallowing tablets, making it easier for them to take the medicine correctly and allowing the dose to be adjusted more precisely than the existing form (which is only available as 50 mg tablets). This makes a major contribution to patient care.

Therefore, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Mercaptopurine Nova Laboratories is of significant benefit for patients affected by acute lymphoblastic leukaemia.

Conclusions

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

Further information on the current regulatory status of Mercaptopurine Nova Laboratories 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.