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Committee for Orphan Medicinal Products

Recommendation for maintenance of orphan designation at the time of addition of a new indication to the marketing authorisation

Soliris (eculizumab) for the treatment of atypical haemolytic uraemic syndrome

During its meeting of 5-7 October 2011, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/09/653 for Soliris (eculizumab) as an orphan medicinal product for the treatment of atypical haemolytic uraemic syndrome. The COMP assessed whether, at the time of addition of a new indication to the 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. 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 extending the approved therapeutic indication for Soliris to include the following indication:

'Atypical hemolytic uremic syndrome (aHUS)'.

This falls within the scope of the product's designated orphan indication(s), which is: 'atypical haemolytic uraemic syndrome'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2009. Atypical hemolytic uremic syndrome remains a condition that is debilitating in the long term or life threatening, particularly due to kidney failure and the risk of the disease coming back despite a kidney or liver transplant.

[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 atypical haemolytic uraemic syndrome 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 0.1 people in 10,000. This is equivalent to a total of around 5,000 people in the EU.

Existence of other satisfactory methods of treatment

The COMP noted that, at the time of the review of the orphan designation, no satisfactory treatments were authorised in the EU for patients affected by this condition.

Conclusions

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

Further information on the current regulatory status of Soliris 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.