27 August 2014
EMA/COMP/340003/2014
Committee for Orphan Medicinal Products

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

Translarna (ataluren) for the treatment of Duchenne muscular dystrophy

During its meeting of 10 to 12 June 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/05/278 for Translarna (ataluren[1]) as an orphan medicinal product for the treatment of Duchenne muscular dystrophy. 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[2].

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

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

'treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older.'

This falls within the scope of the product's designated orphan indication, which is: 'treatment of Duchenne muscular dystrophy'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2005. Duchenne muscular dystrophy remains a condition that is long-term debilitating and life threatening, particularly due to heart and respiratory problems. Patients become gradually wheelchair-bound, and the disease usually leads to death in adolescence or early adulthood.

Prevalence of the condition

The sponsor provided updated information on the prevalence of Duchenne muscular dystrophy based on recently published literature.

[1] Previously known as 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid.
[2] 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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On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of Duchenne muscular dystrophy 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 0.4 people in 10,000. This is equivalent to a total of around 20,000 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 affected by Duchenne muscular dystrophy. Treatment of patients with Duchenne muscular dystrophy primarily involved physiotherapy and other supportive treatments.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Translarna 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 Translarna 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.