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

Brineura (cerliponase alfa) for the treatment of neuronal ceroid lipofuscinosis

On 26 April 2017, the Committee for Orphan Medicinal Products (COMP) completed its review of the designation EU/3/13/1118 for Brineura (cerliponase alfa[1]) as an orphan medicinal product for the treatment of neuronal ceroid lipofuscinosis.[2] 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[3].

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

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

'the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease, also known as tripeptidyl peptidase 1 (TPP1) deficiency.'

This falls within the scope of the product's designated orphan indication, which was originally neuronal ceroid lipofuscinosis type 2 but has been expanded to include all types of the condition.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2013. Neuronal ceroid lipofuscinosis is a seriously debilitating and life-threatening disease that results in progressive brain damage and usually leads to death in the second decade of life.

[1] Previously known as recombinant human tripeptidyl-peptidase 1.
[2] During this review the original orphan indication (neuronal ceroid lipofuscinosis type 2) was expanded to include all types of neuronal ceroid lipofuscinosis.
[3] 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 information on the prevalence of neuronal ceroid lipofuscinosis. On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of neuronal ceroid lipofuscinosis is 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 0.3 people in 10,000. This is equivalent to a total of around 15,000 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 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 Brineura 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 May 2017 COMP minutes.

Further information on Brineura 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.