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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 myasthenia gravis

On 3 July 2017, the Committee for Orphan Medicinal Products (COMP) concluded its review of the designation EU/3/14/1304 for Soliris (eculizumab) as an orphan medicinal product for the treatment of myasthenia gravis. 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 methods of treatment. As other methods of treatment are authorised in the European Union (EU), the COMP also considered whether the medicine is of significant benefit to patients with myasthenia gravis. 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 in adults:

'treatment of refractory generalised myasthenia gravis in patients who are anti-acetylcholine receptor antibody-positive'.

This falls within the scope of the product's designated orphan indication, which is 'treatment of myasthenia gravis'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2014. Myasthenia gravis remains a condition that is long-term debilitating and life threatening due to the weakening of the breathing muscles.

[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 the same therapeutic indication cannot be placed on the market.

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Prevalence of the condition

Following a review of the scientific literature, the sponsor provided an updated estimate of the prevalence of myasthenia gravis, which has increased due to greater awareness and detection 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 myasthenia gravis 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 3.7 people in 10,000. This is equivalent to a total of around 191,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, other treatments were authorised in the EU for the treatment of myasthenia gravis. They included medicines called acetylcholinesterase inhibitors, corticosteroids and medicines that reduce the activity of the immune system (the body's natural defences).

Significant benefit of Soliris

The COMP concluded that the claim of a significant benefit of Soliris in myasthenia gravis is justified because the medicine has been shown to improve symptoms when added to standard treatment in patients whose disease did not respond to at least one previous treatment.

This is based on a study in patients whose disease did not respond to at least one other treatment considered best standard of care such as corticosteroids, immunosuppressant medicines and acetylcholinesterase inhibitors. The majority of patients were also receiving background therapy with a standard treatment. The results showed that treatment with Soliris led to an additional improvement of patients' symptoms and ability to carry out daily activities.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Soliris is of significant benefit to patients affected by myasthenia gravis.

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 and that it should remain in the Community Register of Orphan Medicinal Products.

More information on the COMP assessment can be found in the July 2017 COMP minutes.

Further information on 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.