



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

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

Glybera (alipogene tiparvovec) for the treatment of lipoprotein lipase deficiency

During its meeting of 4-5 September 2012, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/04/194 for Glybera (alipogene tiparvovec, previously known as adeno-associated viral vector expressing lipoprotein lipase) as an orphan medicinal product for the treatment of lipoprotein lipase deficiency. 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. The COMP recommended that the orphan designation of the medicine be maintained<sup>1</sup>.

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

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

'adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein'.

This falls within the scope of the product's designated orphan indication, which is 'treatment of lipoprotein lipase deficiency'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2004. Lipoprotein lipase deficiency remains a condition that is debilitating in the long term and life threatening, particularly due to repeated attacks of pancreatitis.

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<sup>1</sup> 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.



## **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 lipoprotein lipase deficiency 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 0.02 people in 10,000. This is equivalent to a total of around 1,000 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 methods of treatment 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 Glybera still meets the criteria for designation as an orphan medicinal product and that the medicine should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Glybera 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](http://ema.europa.eu/Find%20medicine/Human%20medicines/European%20Public%20Assessment%20Reports).