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Committee for Orphan Medicinal Products

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

## Obizur (susoctocog alfa) for the treatment of haemophilia A

During its meeting of 6 to 8 October 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/10/784 for Obizur (susoctocog alfa[1]) as an orphan medicinal product in the treatment of haemophilia A. 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. 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 haemophilia A. As one of the criteria for orphan designation is no longer met (i.e. the significant benefit), the COMP recommended that the orphan designation of the product should not be maintained[2].

### Life-threatening or chronically debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Obizur for: 'treatment of bleeding episodes in patients with acquired haemophilia caused by antibodies to factor VIII. Obizur is indicated in adults'.

Acquired haemophilia is considered a type of haemophilia A (an inherited bleeding disorder that is caused by the lack of the protein factor VIII). Acquired haemophilia is caused by the spontaneous development of antibodies that inactivate factor VIII. Therefore the approved indication for Obizur is considered to fall within the scope of the product's designated orphan indication, which is: 'treatment of haemophilia A'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2010. Haemophilia A remains a condition that is debilitating in the long term and may be life-threatening because bleeding can occur in the brain, the spinal cord, the throat or the gut.

[1] Previously known as recombinant porcine factor VIII (B-domain-deleted).
[2] The removal of the orphan designation at time of marketing authorisation means that the product cannot benefit from 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 can be placed on the market.

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## Prevalence of the condition

The sponsor provided updated information on the prevalence of haemophilia A based on data from the World Federation of Haemophilia Annual Global Survey of 2011.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of haemophilia A 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 0.7 people in 10,000. This is equivalent to a total of around 36,000 people in the EU.

## Existence of other methods of treatment

At the time of the review of the orphan designation, several medicines were authorised in the EU for the treatment of haemophilia A, including plasma-derived and recombinant factor VIII, to replace the missing factor VIII. However, these medicines do not work in patients who develop antibodies against factor VIII, such as patients with acquired haemophilia. In these cases, other treatments were used, including treatments to try and remove the antibodies from the blood or medicines containing other coagulation factors such as factor VIIa, which attempted to control bleeding by 'by-passing' the use of factor VIII.

## Significant benefit of Obizur

The COMP concluded that the claim of a significant benefit of Obizur in the treatment of haemophilia A is no longer justified because the sponsor has not provided sufficient data to confirm that Obizur will offer a clinically relevant advantage or a major contribution to patient care in patients with haemophilia A.

The sponsor compared data obtained with Obizur with data from published studies for other authorised products for this indication, which did not confirm improved efficacy or safety of Obizur. The sponsor had also claimed that response to treatment could be monitored after injection of Obizur by measuring factor VIII levels, and the dose could be adjusted accordingly. However the COMP concluded that data provided did not support that such dose adjustment would lead to improved bleeding control versus authorised products.

Therefore, the COMP concluded that the assumption that Obizur may be of significant benefit for patients affected by haemophilia A is no longer valid.

## Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP concluded that Obizur no longer meets one of the criteria for designation as an orphan medicinal product. Therefore, the COMP recommended that the product should be removed from the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Obizur 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.