HAUTE AUTORITÉ DE SANTÉ

TRANSPARENCY COMMITTEE

SUMMARY

10 MARCH 2021

The legally binding text is the original French opinion version

turoctocog alfa pegol

ESPEROCT 500 IU, 1,000 IU, 1,500 IU, 2,000 and 3,000 IU, powder and solvent for solution for injection

First assessment

Key points

Unfavourable opinion for reimbursement in the treatment and prophylaxis of bleeding in patients 12 years and above with haemophilia A (congenital factor VIII deficiency).

Role in the care pathway?

The objective of haemophilia A treatment is to achieve early control of and/or to prevent bleeding episodes and their short- and long-term complications, particularly haemophilic arthropathy.

Treatments may be managed using two treatment methods: treatment of bleeding episodes on demand, or continuous or intermittent prophylaxis and/or before a situation promoting bleeding, such as a surgical procedure.

Long-term prophylaxis is the standard treatment in children with severe haemophilia. There is no consensus as to the role of prophylaxis in adults.

Until recently, the only first-line treatment for haemophilia A in the absence of inhibitors was replacement therapy based on the intravenous administration of recombinant or plasma-derived clotting factor VIII concentrates. For the long-term prophylaxis of patients with severe haemophilia A

only, there is now an alternative therapy: emicizumab (HEMLIBRA), a bispecific humanised monoclonal antibody that imitates the clotting function of FVIII and which is administered subcutaneously.

Role of the medicinal product in the care pathway

Considering:
- the demonstration of the efficacy of ESPEROCT (turoctocog alfa pegol) in the treatment and prevention of bleeding in cases of severe haemophilia A in a non-comparative study,
- the lack of data demonstrating a benefit, particularly in terms of efficacy or quality of life, compared to the available alternatives,
- the current state of knowledge, which cannot rule out a risk of the onset of detrimental clinical effects associated with potential PEG accumulation, particularly in the choroid plexus, after numerous years of treatment, a potential risk mentioned in the RMP, in a context in which available alternatives do not present this risk,
- and in view of the numerous therapeutic alternatives available, both for the treatment of bleeding and for long-term prophylaxis, which contribute to coverage of the medical need,

the Committee considers that ESPEROCT (turoctocog alfa pegol) has no role in the treatment and prophylaxis of bleeding in patients 12 years and above with haemophilia A.

COMMITTEE'S CONCLUSIONS

Clinical benefit

◗ Haemophilia A is a constitutional x-linked recessive haemorrhagic genetic disease resulting from coagulation factor VIII deficiency. It is mainly characterised by spontaneous joint bleeding (haemarthrosis) and muscle bleeding (haematoma) or extensive bleeding after an injury. This disease is generally serious, and can be life-threatening.

◗ The proprietary medicinal product ESPEROCT (turoctocog alfa pegol) is a curative and preventive replacement therapy.

◗ The efficacy/adverse effects ratio has not been adequately established in the long term, in view of uncertainties relative to the potential effect of the accumulation of PEG in the choroid plexus of the brain and other tissues or organs, mentioned as an important potential risk in the RMP for ESPEROCT in the same way as other PEGylated FVIII concentrates indicated in haemophilia.

◗ There are medicinal alternatives (see chapter 05).

◗ ESPEROCT (turoctocog alfa pegol) has no role in the care pathway for haemophilia A (see section 08).

Public health impact

Considering:

- the severity of haemophilia A and its low prevalence (rare disease),
- the partially met medical need,
- the lack of response to the partially met identified need in the absence of any demonstrated additional impact on morbidity and mortality, quality of life or the organisation of care,

ESPEROCT (turoctocog alfa pegol) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of ESPEROCT (turoctocog alfa pegol) is insufficient to justify public funding cover in the MA indication in view of the available alternatives.

The Committee issues an unfavourable opinion for inclusion in the hospital formulary list of reimbursed proprietary medicinal products approved for use in the MA indication and dosages.