SUMMARY

efanesoctocog alfa

ALTUVOCT 250 UI, 500 UI, 750 UI, 1000 UI, 2000 UI, 3000 UI and 4000 UI

Powder and solvent for solution for injection

First listing

Adopted by the Transparency Committee on 27 November 2024

Summary of opinion

Favourable opinion for reimbursement in the MA indication: "Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency). ALTUVOCT can be used for all age groups."

Transparency Committee's conclusions

Clinical Benefit

- Haemophilia A is an X-linked, recessive, constitutional, genetic bleeding disorder resulting from a 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.
- This is a curative and preventive substitution therapy.
- The efficacy/adverse effects ratio is high considering, in particular, the results for the efficacy of ALTUVOCT (efanesoctocog alfa) in the XTEND-1 and XTEND-Kids studies as routine weekly prophylaxis, in the treatment of bleeding episodes and during perioperative management, and its favourable safety profile.
- It is a first-line treatment, in the same way as the available alternatives, in the absence of robust comparative clinical data enabling assessment of the benefit of ALTUVOCT (efanesoctocog alfa) compared to these treatments.

Public health benefit

Considering:

- the seriousness of the disease and its prevalence,
- the medical need currently partially met by the available alternatives,
- the lack of response to the identified need considering:
  - the lack of evidence of an additional impact on mortality and morbidity and/or quality of life compared to the available alternatives,

- the lack of evidence of an impact on the organisation of care, and the care and/or life pathway for patients or their families,

ALTUVOCT (efanesoctocog alfa) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of ALTUVOCT 250 IU, 500 IU, 750 IU, 1,000 IU, 2,000 IU, 3,000 IU and 4,000 IU (efanesoctocog alfa) powder and solvent for solution for injection is substantial in the MA indication.

The Committee issues a favourable opinion for inclusion of ALTUVOCT 250 IU, 500 IU, 750 IU, 1,000 IU, 2,000 IU, 3,000 IU and 4,000 IU (efanesoctocog alfa) powder and solvent for solution for injection in the list of covered drugs for inpatients approved for use in the MA indication and at the MA dosages.

Clinical Added Value

Considering:

- efficacy results in the two pivotal studies (XTEND-1 and XTEND-Kids) demonstrating a clinically relevant efficacy of ALTUVOCT (efanesoctocog alfa) as routine weekly prophylaxis, with a mean annualised bleeding rate under treatment of less than 1 in both studies, as well as in the treatment of bleeding episodes and the perioperative management of bleeding,
- the favourable safety profile of ALTUVOCT (efanesoctocog alfa) in these studies, particularly in terms of immunogenicity,
- the favourable pharmacokinetic profile in view of its longer half-life in comparison with other FVIIIs,

but also considering:

- the non-randomised, open-label design of the studies,
- the lack of robust data demonstrating a clinical benefit with ALTUVOCT (efanesoctocog alfa) compared to the available alternatives (FVIII concentrates for the prophylaxis and treatment of bleeding, and emicizumab for prophylaxis only), in terms of either efficacy or safety, given:
  - the methodological limitations of intra-patient comparison of routine prophylaxis with efanesoctocog alfa versus prophylaxis with other FVIIIs (historic data from a previous prospective observational study) conducted in the XTEND-1 study, the only comparative analysis versus the alternatives scheduled in the protocols of the two pivotal studies,
  - the methodological weaknesses of the indirect comparisons provided versus FVIII and emicizumab as prophylaxis,
- the lack of evidence of a benefit on quality of life compared to the available alternatives, particularly for routine prophylaxis in a context of current availability of emicizumab and a FVIII that can be administered every 5 days according to its RCP.

the Committee deems that ALTUVOCT 250 IU, 500 IU, 750 IU, 1,000 IU, 2,000 IU, 3,000 IU and 4,000 IU (efanesoctocog alfa) powder and solvent for solution for injection provides no clinical added value (CAV V) compared to the other FVIII concentrates available.