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Title HEMLIBRA – A Health Technology Assessment

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Reference link to full report in French
https://www.has-sante.fr/jcms/p_3124681/fr/hemlibra

Aim

Assessment of HEMLIBRA (emicizumab) with a view to funding by the French national health insurance system and of its clinical contribution compared to other strategies for the prophylaxis of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) with factor VIII inhibitors.
Hemlibra can be used in all age groups.

Conclusions of Transparency Committee

- The development of FVIII inhibitory antibodies (inhibitors) is currently the main complication of FVIII replacement therapies. In severe haemophilia A, these generally develop from the very first exposures to FVIII, i.e., in the first years of the patient's life. This complication has a major impact in terms of threat to life, functional performance and quality of life, particularly for high responder patients.
- It is a preventive treatment.
- The efficacy/adverse effects ratio is high in high responders.
- There are therapeutic alternatives for prophylaxis (FEIBA and NOVOSEVEN).
- It is a first-line treatment in high responders requiring long-term prophylaxis.
- HEMLIBRA is likely to have an impact on public health.

Given these elements, the clinical benefit provided by HELIMBRA is **substantial in the prophylaxis of bleeding episodes only** in patients with congenital haemophilia A who have developed a high-responding factor VIII inhibitor,

Considering:

- the partially met medical need for the management of patients with congenital haemophilia A with high-responding inhibitors,
- phase 3 clinical trials conducted exclusively in patients having developed a high-responding inhibitor, predominantly with a severe form of haemophilia,
- data demonstrating the efficacy of HEMLIBRA to prevent bleeding in this population, particularly in children,
- data suggesting a superior efficacy to that of prophylaxis with bypassing agents,
- the substantial benefit expected on quality of life compared to the available alternatives,
- and despite uncertainties relative to its long-term use and in routine practice (impact of the drug interaction with FEIBA on the prognosis of patients treated for serious breakthrough bleeding, management of surgical procedures, use in the oldest patients, those with coronary artery disease or bleeding little, risk of anti-emicizumab inhibitory antibodies, interference with certain coagulation tests),

HEMLIBRA provides an important clinical added value (CAV II) compared to bypassing agents (FEIBA and NOVOSEVEN) in the management of patients with congenital haemophilia A having developed a high-responding factor VIII inhibitor.

Recommendations

The Transparency Committee issued its approval for the funding of HEMLIBRA by the French national health insurance system for the prophylaxis of bleeding episodes in patients with congenital haemophilia A who have developed a high-responding factor VIII inhibitor.

Methods

The assessment of HEMLIBRA was founded on evidence-based medicine with a critical analysis of the clinical data.

Written by

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