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Media and Public Relations

Press release

First-in-class medicine to prevent bleeding in haemophilia A patients with inhibitors

Hemlibra recommended for approval following accelerated assessment

The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended granting a marketing authorisation for Hemlibra (emicizumab), a first-in-class medicine to prevent bleeding or reduce the frequency of bleeding episodes in patients with haemophilia A with factor VIII inhibitors, in patients of all ages.

Haemophilia A is an inherited bleeding disorder caused by lack of a clotting protein called factor VIII, and affects mainly males. Patients with haemophilia A are usually treated with factor VIII medicines, which replace the missing factor VIII and help control and prevent bleeding. However the body may develop inhibitors (antibodies) as a reaction to these medicines. The inhibitors reduce the medicines' effect, so bleeding is no longer controlled.

The development of inhibitors is the most severe treatment-related complication of haemophilia A because it makes it difficult to manage the disease. Current treatment alternatives in patients with haemophilia A who develop inhibitors are time-consuming and often burdensome, particularly for children, and they are not effective in all patients. There is therefore an unmet medical need for more convenient and effective treatment options.

Hemlibra is the first monoclonal antibody to be recommended for use in patients with haemophilia A with inhibitors, an area of medicine where no new medicines have been made available in 20 years. It works by mimicking the coagulation function of factor VIII. The treatment is given weekly via a subcutaneous injection, making it more convenient than bypassing agents (medicines that bypass factor VIII) which are the current standard of care but which require frequent, prolonged administration by infusion (drip). The CHMP reviewed the application for Hemlibra under its accelerated assessment procedure, which allows the speeding up of patients' access to medicines that address unmet medical needs.

The safety and efficacy of the medicine was evaluated in two phase III clinical trials: a randomised, open-label study conducted in 109 patients aged 12 years or older, and an ongoing single-arm, open-label study in children under 12 years of age, for which results in 60 patients were included in the application. Overall, the prophylactic use of emicizumab in haemophilia A patients with inhibitors

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reduced bleeding episodes that needed treatment with coagulation factors by around 80-90% compared to on-demand use of bypassing agents without prophylactic treatment.

The most common adverse events observed were reactions at the site of injection, headache, thrombotic microangiopathy (damage to small blood vessels supplying organs such as the kidney), fever, diarrhoea and joint and muscle pain.

The opinion adopted by the CHMP is an intermediary step on Hemlibra's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Hemlibra is Roche Registration Limited.
3. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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