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Press Office

Press release

First specific replacement therapy to treat rare bleeding disorder

Coagadex recommended for approval for prevention of bleeding episodes for patients with factor X deficiency

The European Medicines Agency (EMA) has recommended granting a marketing authorisation for Coagadex (human coagulation factor X) to treat factor X deficiency, a rare inherited bleeding disorder.

Coagadex is indicated for the treatment and preventive management of bleeding episodes and the control of bleeding during surgical procedures in patients with hereditary factor X deficiency. The medicine's active substance is human coagulation factor X, a protein derived from human plasma.

This disorder is caused when the body does not produce enough factor X, which is needed for the blood coagulation (clotting) process. Patients with this condition are more prone to bleeding than other people and show prolonged bleeding after injury or surgery. Bleeding can also occur within muscles or the spaces in the joints, such as the elbows, knees and ankles, which can lead to permanent injury if it happens repeatedly. Factor X deficiency is a debilitating, lifelong disease and may be life threatening if bleeding occurs in the brain, the spinal cord or the gut. The disease can vary in severity (from mild to severe) and affects men and women equally.

Current treatment for this condition includes replacement therapies, which contain a mix of coagulation factors; these are associated with problems with dosing and the risk of elevating other clotting factors which may result in complications. Due to the lack of specific treatment options, EMA's Committee for Medicinal Products for Human Use (CHMP) decided to speed up the evaluation of this medicine and recommended marketing authorisation following an accelerated assessment. This mechanism is one of the Agency's tools to speed up patient access to new medicines if they address an unmet medical need.

The CHMP based its recommendation on the results of two non-randomised, open-label, multi-centre studies. Since factor X deficiency is a rare disease the number of participants in these studies was small. A Phase 3 study evaluated the treatment of a total of 207 spontaneous bleeds in 16 patients with moderate or severe factor X deficiency and demonstrated an effective response (excellent or good in 98.8% of the bleeds treated). Five patients participating in the study underwent seven surgical procedures and Coagadex was also shown to be effective for the prevention of bleeding during planned surgery. An additional study in children with mild to severe hereditary factor X deficiency is ongoing.

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The CHMP also determined that despite the limited safety information available due to the rarity of the disease, the adverse event profile appeared acceptable. The most common side effects with Coagadex were infusion-site erythema (superficial reddening of the skin), infusion-site pain, back pain and fatigue.

The applicant received scientific advice on the design of the trials from the CHMP. Scientific advice is one of the Agency's main tools to facilitate and stimulate research and development within the European Union (EU).

Because factor X deficiency is rare, Coagadex was designated as an orphan medicine by the Committee for Orphan Medicinal Products (COMP). Orphan designation gives medicine developers access to incentives such as fee reductions for scientific advice, and is the key instrument available in the EU to encourage the development of medicines for patients with rare diseases.

The opinion adopted by the CHMP at its January 2016 meeting is an intermediary step on Coagadex'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 Coagadex is Bio Products Laboratory.
3. Following this positive CHMP opinion, the COMP will assess whether the orphan designation should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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