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Questions and answers

Questions and answers on the outcome of extension of indication application for Kogenate Bayer and Helixate Nexgen (octocog alfa)

On 15 March 2012, the Committee for Medicinal Products for Human Use (CHMP) finalised the assessment of an application to add immune tolerance induction as a new indication for Kogenate Bayer and Helixate Nexgen (octocog alfa). The CHMP did not consider the data submitted by the company to be sufficient to recommend the new indication for these medicines. However, the Committee concluded that the new data could be included in the product information for Kogenate Bayer and Helixate Nexgen, as they might be of interest to healthcare professionals using the medicines.

What are Kogenate Bayer and Helixate Nexgen?

Kogenate Bayer and Helixate Nexgen are medicines containing the active substance recombinant human coagulation factor VIII (octocog alfa). They are used for the treatment and prevention of bleeding in patients with haemophilia A (an inherited bleeding disorder). Patients with haemophilia A lack factor VIII and these medicines work by 'replacing' the missing factor VIII. They are given by injection.

What were Kogenate Bayer and Helixate Nexgen expected to be used for?

When a haemophilia A patient is treated with factor VIII, the body may start producing antibodies against it, which can cause the medicine to stop working. In addition to their existing indication, Kogenate Bayer and Helixate Nexgen at a higher dose were expected to be approved to treat these patients who have developed antibodies against factor VIII. The treatment was designed to make the patients tolerant again to factor VIII (also called immune tolerance induction) and stop the immune system from producing antibodies so that the treatment for haemophilia remains effective.

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What did the company present to support its application?

The company presented data from a study involving 39 haemophilia A patients with antibodies against factor VIII, who were treated with Kogenate Bayer and Helixate Nexgen at different doses and for a variable duration of time. The company also provided results from a review of 32 patients. The patients' blood levels of antibodies were measured and the main measure of effectiveness was the proportion of patients whose blood was cleared of antibodies.

What was shown in the study?

Kogenate Bayer and Helixate Nexgen successfully eliminated antibodies from the blood in 46.2 % (6 patients out of 13 patients that were evaluated) in the first study and 68.8 % of patients (22 out of 32 patients) in the second study.

What was the conclusion of the CHMP?

The CHMP noted that high doses of factor VIII have been used for several decades to induce tolerance in patients producing antibodies against factor VIII. However, there was no standardised and approved dose regimen for this use. The two submitted studies reflected the current clinical use in accordance with the approved product information, but did not provide new information supporting the indication applied for. The first study which was designed to provide data and guidance on how to use factor VIII to induce tolerance was terminated early. Many uncertainties remained on, for example, the appropriate dose for a patient based on the level of antibodies in their blood, the treatment schedule and the appropriate duration of treatment. The Committee concluded that the data were insufficient to approve immune tolerance induction as an indication for Kogenate Bayer and Helixate Nexgen.

However, the Committee concluded that some data provided by the studies could be added to the product information for Kogenate Bayer and Helixate Nexgen, as they might be of interest to healthcare professionals using the medicines.

The full European Public Assessment Reports for Kogenate Bayer and Helixate Nexgen can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports.