20 May 2022
EMA/267926/2022
EMEA/H/C/005547

Withdrawal of application for the marketing authorisation of HemAryo (eptacog alfa (activated))

UGA Biopharma withdrew its application for a marketing authorisation of HemAryo for treating bleeding episodes and for preventing bleeding after surgical procedures in patients with clotting disorders.

The company withdrew the application on 6 May 2022.

What is HemAryo and what was it intended to be used for?

HemAryo was developed as a medicine for treating bleeding episodes and for preventing bleeding after surgery or invasive procedures in patients who have:

- congenital haemophilia (a bleeding disorder present from birth) who have developed or are expected to develop 'inhibitors' (antibodies) against factor VIII or IX (proteins involved in blood clotting);
- acquired haemophilia (a bleeding disease caused by the development of inhibitors to factor VIII);
- congenital factor VII deficiency;
- Glanzmann's thrombasthenia (a rare bleeding disorder) who cannot be treated with a transfusion of platelets (components that help the blood to clot).

HemAryo contains the active substance eptacog alfa and was to be available as powder and solvent that are made up into a solution to be injected into a vein.

HemAryo was developed as a 'biosimilar' medicine. This means that HemAryo was intended to be highly similar to another biological medicine already authorised in the EU (the 'reference medicine'). The reference medicine for HemAryo is NovoSeven. For more information on biosimilar medicines, see here.

How does HemAryo work?

The active substance in HemAryo, eptacog alfa, is almost identical to a human protein called factor VII. Eptacog alfa works in the same way as factor VII. In the body, factor VII is involved in blood clotting. It activates another factor called factor X, which starts the clotting process. By activating factor X, eptacog alfa is able to give temporary control of the bleeding disorder.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

Because factor VII acts directly on factor X, independently from factors VIII and IX, eptacog alfa can be used in haemophilia patients who have developed inhibitors to factor VIII or IX, and to replace the missing factor VII in patients with factor VII deficiency.

What did the company present to support its application?

The company presented results from laboratory studies comparing HemAryo with the reference medicine, to investigate whether the active substance in HemAryo is highly similar to that in NovoSeven in terms of structure, purity and biological activity.

Results from two clinical studies were also presented to show that HemAryo produces similar levels of the active substance to the reference medicine in the body, and that its effectiveness could be comparable to that of NovoSeven.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the initial information from the company and had prepared questions for the company. The company had not responded to the questions at the time of the withdrawal.

What did the Agency recommend at that time?

Based on the review of the data, at the time of the withdrawal, the Agency had some concerns and its provisional opinion was that HemAryo could not have been authorised for the treatment of bleeding episodes and for the prevention of bleeding after surgical procedure in patients with clotting disorders.

The Agency had concerns about the way the medicine was produced, leading to uncertainties about the quality of the medicine. The Agency also had concerns about the design and conduct of the studies comparing HemAryo with the reference medicine.

Therefore, at the time of the withdrawal, the Agency was not able to draw conclusions on whether HemAryo is highly similar to the reference medicine, and concluded that the medicine could not have been authorised based on the data from the company.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of the application, the company stated that it was withdrawing the application due to major manufacturing issues.

Does this withdrawal affect patients in clinical trials?

The company informed the Agency that all clinical trials of HemAryo have been completed.