論 文 要 旨

A phase III clinical trial of a mixture agent of plasma-derived factor VIIa and factor X (MC710) in haemophilia patients with inhibitors

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Abstract

Introduction:

MC710, a 1:10 protein weight ratio mixture of plasma-derived activated factor VII (FVIIa) and factor X (FX), is a novel bypassing agent for haemostasis in haemophilia patients with inhibitors. We evaluated the haemostatic efficacy and safety of one to two administrations of MC710 in 21 joint, muscle, and subcutaneous bleeding episodes in 14 male patients, in a multi-centre, open-label, non-randomized clinical trial.

Methods:

Subjects were intravenously administered one or two doses of 60 or 120 μg kg^{-1} MC710 (as FVIIa) once or twice (to a maximum of 180 μg kg^{-1}) over up to five bleeding episodes per subject. The haemostatic efficacy of MC710 was determined for each episode by investigator evaluation, using changes in visual analogue scale (VAS) for pain relief, and/or knee joint or muscle circumference for swelling reduction, and range of motion (ROM) for improvement of joint mobility.

Results:

In 21 treatments for bleeding episodes, 19 were rated “excellent” or “effective” 8 h after the last treatment. VAS significantly decreased over time, and ROM significantly improved over time compared with the values before treatment. One mild adverse reaction, decreased blood potassium, and two serious adverse events, both knee joint bleeding, were observed within 1 week after first administration, with no significant effect on safety. Furthermore, diagnostic markers did not show any signs of disseminated intravascular coagulation (DIC).

Conclusion:

These results show that MC710 has sufficient haemostatic efficacy and safety, and can be used as a potential bypassing agent to control bleeding in haemophilia patients with inhibitors.

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