NEW DRUGS

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Efmoroctocog alfa

Approved indication: haemophilia A

Eloctate (Bioverativ)
vials containing 250 IU, 500 IU, 750 IU, 1000 IU, 1500 IU, 2000 IU and 3000 IU
Australian Medicines Handbook: Appendix A

Haemophilia A is an inherited X-linked disorder that results in a deficiency of clotting factor VIII. A patient's risk of bleeding is influenced by the severity of the deficiency. To prevent or control bleeding the patient is given intravenous factor VIII. Nowadays a genetically engineered product is used in preference to products derived from plasma.

The half-life of some recombinant factor VIII products is 8–12 hours. This means that patients will need several injections a week to maintain an effective concentration of factor VIII. In contrast, the half-life of efmoroctocog alfa is 19 hours, so less frequent dosing may be possible.

Efmoroctocog is a genetically engineered molecule consisting of factor VIII linked to part of a human immunoglobulin (IgG Fc domain). The immunoglobulin component of this fusion protein binds to receptors in many cells and this delays the degradation of the factor VIII component.

The drug was first tested in 16 patients with severe haemophilia A. They were given a single dose of recombinant factor VIII and then a dose of efmoroctocog a few days later. Both drugs increased factor VIII activity, but efmoroctocog had a lower clearance. A more sustained response could be predicted.[1]

To test the efficacy and safety of efmoroctocog, 165 patients, aged 12 years and above, were enrolled in an open-label trial. These patients all had severe haemophilia A and had bled at least 12 times in the previous year. They were managed either with an individualised prophylactic regimen given every 3–5 days (118 patients), weekly prophylaxis (24 patients) or episodic treatment for bleeding (23 patients). The median duration of treatment was approximately 30 weeks. Approximately 45% of the patients taking individualised prophylaxis had no bleeding. Their annual bleeding rate was 2.9 compared with 8.9 for weekly prophylaxis and 37.3 for episodic treatment.[2]

During the phase III trial adverse events related to treatment occurred in 6.1% of the patients. These were most commonly arthralgia and malaise. No patients developed inhibitors to factor VIII.[2]

While efmoroctocog is eliminated more slowly than some other recombinant factor VIII products, it also takes longer for its effects to begin.[1] Whether the differences between the molecules lead to significant differences in clinical outcomes remains to be seen.

T T manufacturer provided additional useful information

REFERENCES

1. Powell JS, Josephson NC, Quon D, Ragni MV, Cheng G, Li E, et al. Safety and prolonged activity of recombinant factor VIII Fc fusion protein in hemophilia A patients. Blood 2012;119:3031-7. https://doi.org/10.1182/blood-2011-09-382846
2. Mahlangu J, Powell JS, Ragni MV, Chowdary P, Josephson NC, Pabinger I, et al. Phase 3 study of recombinant factor VIII Fc fusion protein in severe hemophilia A. Blood 2014;123:317-25. https://doi.org/10.1182/blood-2013-10-529974

The Transparency Score is explained in New drugs: transparency, Vol 37 No 1, Aust Prescr 2014;37:27.

At the time the comment was prepared, information about this drug was available on the website of the Therapeutic Goods Administration.