Evaluate Vantage

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Roche finds safe Haven in emicizumab

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Roche’s bad news on the multiple sclerosis project Ocrevus was countered today with good news in haemophilia A with emicizumab, which showed that it can reduce bleeding incidents in patients who have developed resistance to first-line treatment.

The antibody appears to have withstood safety worries related to thrombotic events, as the four reported cases have been linked to emicizumab’s use with bypassing agents. If regulators approve it, emicizumab’s advantages in efficacy and dosage should allow it to become a blockbuster, one of only two in the space, and put Shire’s haemophilia franchise on the back foot.

Wait until June?

The Swiss group did not release specific data from the Haven 1 trial beyond the topline announcement that the primary and secondary endpoints had all been met when emicizumab was used as a preventive therapy in combination with episodic treatment for control of bleeding incidents.

The main findings were that patients taking emicizumab had significantly fewer bleeding incidents over 24 weeks than those using only the bypassing agents episodically. Those on episodic treatment alone were given emicizumab after the first 24 weeks.

A third arm included patients who had been receiving bypassing agents preventively before entering the trial, and on entry into the study got emicizumab alone preventively. Those patients saw a significant decline in bleeding rates after initiating emicizumab treatment, Roche said.

Analysts from Berenberg expect full data at the International Society on Thrombosis and Haemostasis in Berlin in July.

The positive data show the progress being made by bispecific antibodies, of which emicizumab is an example. The progress was further confirmed yesterday when Incyte paid $120m and invested another $80m in Merus for up to 11 bispecific projects in oncology.

The risk factor

The safety findings disclosed by Roche were reassuring – thrombosis, after all, is a risk of drugs designed to stimulate blood clotting, as demonstrated by the warnings for Shire’s Feiba bypassing factor, for example.

Roche had previously announced that two patients taking emicizumab had thrombotic events and two others developed thrombotic microangiopathy – in all four cases the patients had been treated with prothrombin complex concentrate to treat breakthrough bleeds.

Neither patient who developed thromboembolisms needed anti-coagulation therapy, and one resumed emicizumab. The patients with thrombotic microangiopathy saw their condition resolve, and one began taking emicizumab again.

Bernstein analyst Tim Anderson suggested that thrombosis could be handled through warnings or a black box on the label, although whether that is the case will become clearer only after the scientific community and regulators have a closer look at the data.

The regulators will most likely have to make quick work of it. Emicizumab has US breakthrough therapy designation, which qualifies it for rolling submission and priority review. This was based on its effectiveness in haemophilia patients who have developed resistance.

Roche has stated that it expects to launch emicizumab next year in this population, which puts it on track to achieve $1.5bn in US and Europe sales by 2022. The only thing that could stop this is if regulators want a better understanding of drug interactions that raise thrombotic risks.

Study	Trial ID
Haven 1	NCT02622321

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