Name of drug: Emicizumab, also known as ACE910

Who is it for? People with hemophilia A (factor VIII deficiency) with an inhibitor. Potentially for those without inhibitors also.

Who is developing it? Roche, the world's largest biotech company. Genentech is Roche's company in the U.S. The other company involved is Chugai Pharmaceutical of Japan, of which Roche is the majority shareholder.

What is the status? Emicizumab is being evaluated in phase III clinical trials in people 12 years of age and older, both with and without inhibitors to factor VIII, and in children under 12 years of age with factor VIII inhibitors. On August 24, 2017, the FDA granted Priority Review for emicizumab prophylaxis as a once-weekly subcutaneous treatment for adults, adolescents and children with hemophilia A with an inhibitor. Priority Review designation is granted to medicines that the FDA has determined to have the potential to provide significant improvements in the safety and effectiveness of the treatment, prevention or diagnosis of a serious disease. This regulatory review fast track means Genentech will only have to wait 6 months, rather than the usual 10, to learn what the FDA makes of the risk-benefit profile of the drug. The FDA is expected to make a decision on approval by February 23, 2018.

Additional studies evaluating emicizumab in people with hemophilia A both with and without inhibitors and exploring less frequent dosing regimens are ongoing. In 2018, Genentech plans to submit the drug to the FDA for approval in hemophilia A patients without inhibitors.

What is it? Emicizumab is a monoclonal antibody designed to bring together factors IXa and X, proteins that are needed to activate the natural coagulation cascade and restore the blood clotting process. Emicizumab can be administered by an injection of a ready-to-use solution under the skin (subcutaneously) once weekly.

What are the potential benefits?

- In their latest clinical trial report, Genentech said emicizumab cut the bleed rate by 87 percent in inhibitor patients compared with those who received another treatment. After a median observation time of 31 weeks, 62.9% of patients receiving emicizumab experienced zero treated bleeds compared to 5.6% of those receiving on-demand by-passing agents (either NovoSeven or FEIBA).
- Results also showed a statistically significant and clinically meaningful improvement in health-related quality of life.
- Interim results from the study in children younger than 12 years of age with inhibitors who received emicizumab prophylaxis are consistent with the positive results from the adult/adolescent trial. After a median observation time of 12 weeks, the study showed that only one of 19 children receiving emicizumab reported a treated bleed. There were no reported joint or muscle bleeds. The data also indicate that the same dose of emicizumab is appropriate for children as for adults and adolescents.

- Inhibitor patients who currently are injecting NovoSeven or FEIBA intravenously multiple times a week could inject emicizumab subcutaneously once per week.

What adverse reactions have been reported?

Adverse events (AEs), occurring in 5% or more of patients treated, were:
mild injection site reactions
headache
fatigue
common cold symptoms (nasopharyngitis)
joint pain.

Serious adverse events were:
thromboembolic events (blood clots) – two patients
thrombotic microangiopathy (clots in the small blood vessels inside vital organs) - three patients
These adverse events were associated with repeated high doses of FEIBA, used to treat breakthrough bleeds. The Genentech representative who spoke to HoG staff said he expects there to be a warning about the concurrent use of FEIBA and emicizumab. Shire, the manufacturer of FEIBA, and which stands to lose market share to emicizumab, has called Genentech's explanation for the adverse events "inaccurate and misleading."