

THROMBOTIC MICROANGIOPATHY CASES

Important Safety Information & Indication

Indication

HEMLIBRA® is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII, and the dose and schedule to use for breakthrough bleed treatment. HEMLIBRA may cause the following serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including:

- **Thrombotic microangiopathy (TMA).** This is a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:

- Confusion
- Weakness
- Swelling of arms and legs
- Yellowing of skin and eyes
- Stomach (abdomen) or back pain
- Nausea or vomiting
- Feeling sick
- Decreased urination

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

You may report side effects to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Genentech at (888) 835-2555, 24 hrs/day, 7 days/week.



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THROMBOTIC MICROANGIOPATHY CASES IN EMICIZUMAB-KXWH CLINICAL TRIALS, EXPANDED ACCESS, COMPASSIONATE USE, AND AFTER FDA APPROVAL^{1,2}

- Review of the thrombotic microangiopathy (TMA) adverse events in the emicizumab-kxwh clinical trial program found these events were reported when on average a cumulative amount of >100 U/kg/24 hours of aPCC (FEIBA®) was administered for 24 hours or more to people receiving emicizumab-kxwh prophylaxis.
- **As of June 30, 2020:**
 - No TMA cases have occurred in the expanded access program or through compassionate use requests.¹
 - 3 cases of TMA occurred in the HAVEN 1 study and 1 case occurred after the FDA approval of emicizumab-kxwh. HAVEN 1 was a Phase 3 clinical trial in adults and adolescents ≥12 years of age with hemophilia A with factor VIII inhibitors.²

Thrombotic Microangiopathy Cases Reported/Verified at Data Cutoff of June 30, 2020^{1,2}

TMA Cases from Clinical Trials, Expanded Access, and Compassionate Use		
Number of cases	Cases exceeded on average a cumulative amount of >100 U/kg/24h of aPCC for 24h or more*	Clinical Trial Where Event Occurred
3 cases	3 cases	HAVEN 1 (Australia, Europe, North America)

TMA Cases after FDA Approval		
Number of cases	Cases exceeded on average a cumulative amount of >100 U/kg/24h of aPCC for 24h or more*	Where Events Occurred
1 case	1 case	North America

*See the emicizumab-kxwh [Medication Guide](#) including, "What is the most important information I should know about HEMLIBRA?"

Patient safety is of the highest importance to us. We take all reports of safety events very seriously and encourage anyone who knows of a side effect in a patient on emicizumab-kxwh to report the event to Genentech/Roche. We have systems and processes in place to collect, analyze, and monitor side effects and report events to the FDA per regulations.

Due to the voluntary nature of postmarketing spontaneous side effect reports, information may be missing or incomplete. Genentech/Roche has limited ability to ascertain and verify information from these side effect reports, and reporters, including healthcare providers, are not obligated to share these details with Genentech/Roche. Furthermore, reporters themselves may not have access to all of the information regarding a patient's care for these events. Genentech/Roche does not provide additional details related to side effects reported in the postmarketing setting, because the level of detail available and Genentech/Roche's ability to confirm individual details is variable. In addition, patient privacy is very important to Genentech/Roche, therefore we are careful not to disclose specific details about a side effect that could jeopardize the privacy of either the patient or their family, or breach patient confidentiality. As a result of the variable level of detail in such spontaneously reported data, Genentech/Roche will provide information on the number of verified reports on this website without assessments of relatedness or additional reported details related to events.

If any side effect in a person taking emicizumab-kxwh impacts the overall safety profile of the medicine, we will share this information as quickly as possible and in accordance with any FDA requirements.

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

Your healthcare provider should be the primary source of information about your medical condition and the safe and effective use of any medicine, including emicizumab-kxwh.

You may contact our Medical Communications department with questions specific to this site: (800) 821-8590, 5:00A – 5:00P PST.

References

1. Data on file, Genentech, Inc. June 2020;
2. Oldenburg J et al. *N Engl J Med.* 2017;377:809-818.