USFDA gives nod to Pfizer's drug therapy for patients with Hemophilia

The FDA granted Hympavzi Orphan Drug designation for this application.

The FDA granted the approval of Hympavzi to Pfizer Inc. (Image Credits: Pixabay)

The U.S. Food and Drug Administration (USFDA) approved Hympavzi (marstacimab-hncq) for patients suffering from Hemophilia A or B. According to a press statement, the approval has been granted for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients 12 years of age and older with hemophilia A without factor VIII inhibitors or hemophilia B without factor IX inhibitors (neutralizing antibodies).

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"Today's approval of Hympavzi provides patients with hemophilia a new treatment option that is the first of its kind to work by targeting a protein in the blood clotting process," said Ann Farrell, M.D., Director of the Division of Non-Malignant Hematology in the FDA's Center for Drug Evaluation and Research. "This new type of treatment underscores the FDA's commitment to advance the development of innovative, safe and effective therapies."

Hemophilia A and hemophilia B are genetic bleeding disorders caused by a dysfunction or deficiency of coagulation factor VIII (FVIII) or IX (FIX), respectively. Patients with these hemophilias are unable to clot properly and may bleed for a longer time than normal after injury or surgery. They may also have spontaneous bleeding in muscles, joints and organs, which can be life-threatening. These bleeding episodes are typically managed by either on-demand, episodic treatment or prophylaxis using products containing FVIII or FIX, or a product that mimics a factor.

According to the USFDA, Hympavzi is a new type of drug that, rather than replacing a clotting factor, works by reducing the amount, and therefore, the activity of, the naturally occurring anticoagulation protein called tissue factor pathway inhibitor. This increases the amount of thrombin, an enzyme that is critical in blood clotting, that is generated. This is expected to reduce or prevent the frequency of bleeding episodes.

Hympavzi's approval is based on an open-label, multi-center study in 116 adult and pediatric male patients with either severe hemophilia A or severe hemophilia B, both without inhibitors, USFDA stated.

Hympavzi comes with warnings and precautions about circulating blood clots (thromboembolic events), hypersensitivity and embryofetal toxicity. The most common side effects of Hympavzi are injection site reactions, headache and itching (pruritis), the US regulatory body said. The FDA granted Hympavzi Orphan Drug designation for this application.

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