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FDA OKs Baxalta's drug for von Willebrand bleeding disorder

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U.S. regulators on Tuesday approved the first genetically engineered treatment for the most common inherited bleeding disorder, von Willebrand disease.

The Food and Drug Administration said that it's approved Vonvendi, made by Baxalta Inc., for treating patients aged 18 and older.

According to the FDA, von Willebrand disease affects about 1 percent of the U.S. population, or more than 3 million Americans.

The disease is caused by a defect or deficiency in a protein critical for normal blood clotting, von Willebrand factor.

Symptoms can include severe bleeding from the nose, gums, intestines, muscles and joints. In women, it can cause very heavy and prolonged menstrual periods and excessive bleeding after childbirth. Bleeding episodes after an internal injury or cut can damage joints and organs.

Von Willebrand disease is generally less severe than hemophilia. Most people with von Willebrand have type 1, the mildest of the three types, and may only need treatment to prevent bleeding before surgery or tooth extraction. Patients with more severe forms need emergency treatment to stop bleeding before it becomes life-threatening, but can live normal lives with proper treatment, according to the National Heart, Lung and Blood Institute.

Dr. Karen Midthun, director of the FDA's Center for Biologics Evaluation and Research, said Vonvendi's approval gives patients an additional therapy for treating bleeding episodes.

Vonvendi should be available sometime next year. A Baxalta spokesman said the drug's price has not yet been set.

Baxalta, which develops treatments for bleeding disorders and other conditions, is the former biopharmaceutical business of Baxter International Inc., which spun off the unit earlier this year. Baxalta is based in Bannockburn, in northern Illinois.

Baxter makes medical products including drug delivery systems, anesthesia and surgery products, and intravenous solutions.