FDA approves first gene therapy for severe hemophilia A

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The U.S. Food and Drug Administration on Thursday approved a costly

single-dose gene therapy for patients with severe hemophilia A, a life-threatening hereditary bleeding disorder.

The treatment is not cheap: Roctavian will cost $2.9 million for a single infusion, the *Associated Press* reported.

"Hereditary hemophilia A is a potentially serious bleeding disorder. Severe cases of hemophilia A can cause life-threatening health issues due to increased risk of uncontrolled bleeding," Dr. Peter Marks, director of the FDA's Center for Biologics Evaluation and Research, said in an agency news release. "Today's approval represents an important advance in providing treatment options for patients with this bleeding disorder, and treatment with gene therapy may reduce the need for ongoing routine therapy."

For patients, this means a potential reduction in uncontrolled bleeding. Left untreated, severe hemophilia A can cause life-threatening bleeding into vital organs, including the kidneys and brain.

Drug maker BioMarin Pharmaceutical Inc. told the *AP* that the drug's astronomical price tag reflects "the possibility of freedom from years" of infusions with other medications. Those infusions typically run about $800,000 each year for patients, the *AP* said.

Hemophilia A is caused by a mutation on a gene which produces a protein that enables blood to clot. This protein is called Factor VIII. The condition primarily affects males. How frequent and severe bleeding episodes are depends on the amount of FVIII protein a person produces. In about 60% of cases, people with severe hemophilia A have less than 1% of FVIII in their blood.

Before now, treatment has involved FVIII replacement therapy or an antibody-based medication, the FDA said.

Roctavian is a viral vector that carries the gene for Factor VIII. It reduces the risk of uncontrolled bleeding by delivering the gene into the liver, to increase blood levels of FVIII.

The approval was based on a multinational study in adult men ages 18 to 70 who had previously had FVIII replacement therapy determined the drug's safety and effectiveness. A total of 112 patients were followed for at least three years.

Those treated in the study had far fewer bleeding incidents. Their mean annualized bleeding rate dropped from 5.4 times each year to 2.6. Most of those who received Roctavian also took corticosteroids to suppress their immune system. The FDA noted that treatment response may decrease over time.

Adverse reactions seen in the study included mild changes in liver function, headache, nausea, vomiting, fatigue, abdominal pain and infusion-related reactions.

The treatment is not without risks. The FDA recommends close monitoring for infusion-related reactions and elevated liver enzymes. An increase in FVIII activity could potentially increase the risk of blood clots, the FDA noted. The product also carries a potential cancer risk.

More information: The National Hemophilia Association has more on hemophilia A.