FDA APPROVES FIRST GENE THERAPY FOR HEMOPHILIA B

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The Food and Drug Administration approved the first gene therapy for adults with hemophilia B who are currently being treated with Factor IX prophylaxis and have a history of repeated, serious bleeding episodes. Etranacogene dezaparvovec (Hemgenix, CSL Behring) is a one-time adeno-associated virus vector-based therapy that works by carrying a gene for clotting Factor IX. The gene is expressed in the liver to produce Factor IX protein, which increases blood levels of Factor IX and helps to limit bleeding episodes.

Investigators conducted two studies of 57 adult men with severe or moderately severe hemophilia B to evaluate the therapy's safety and efficacy. Effectiveness was established based on decreases in the men's annualized bleeding rate (ABR). In one study with 54 participants, the subjects had increases in Factor IX activity levels, a decreased need for routine Factor IX replacement prophylaxis, and a 54% reduction in ABR compared with baseline.

The most common adverse reactions associated included liver enzyme elevations, headache, mild infusion-related reactions and flu-like symptoms. Patients should be monitored for adverse infusion reactions and liver enzyme elevations (transaminitis) in their blood.

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