FDA approves first gene therapy to treat Duchenne muscular dystrophy

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The U.S. Food and Drug Administration on Thursday approved the drug

Elevidys (delandistrogene moxeparvovec-rokl), the first gene therapy for the treatment of children with Duchenne muscular dystrophy (DMD).

The groundbreaking treatment will not be cheap: Drugmaker Sarepta Therapeutics Inc. said it would charge $3.2 million for the one-time intravenous treatment, the *Associated Press* reported. Like most medicines in the United States, the cost would be mostly paid by insurers. The drug will be used to treat children ages 4 through 5 who have a mutation in the DMD gene.

"The approval of Elevidys is a watershed moment for the treatment of Duchenne. Elevidys is the first and only gene therapy approved for Duchenne, and this approval brings us closer to our goal of bringing forward a treatment that provides the potential to alter the trajectory of this degenerative disease," Sarepta President and CEO Doug Ingram said in a company statement.

Most current treatments target symptoms, not the underlying genetic cause of the debilitating condition. But Elevidys is a recombinant gene therapy that delivers a gene that boosted production of Elevidys micro-dystrophin, a protein that contains a dystrophin protein seen in normal muscle cells. The product is given in a single intravenous dose.

In approving the drug, the FDA considered the risks associated with the drug, the life-threatening and debilitating nature of the disease for children, and the urgent unmet medical need, the agency said. A clinical benefit of Elevidys, including improved motor function, has not been established. As a condition of approval, the FDA has required the company to complete a study to confirm the drug's clinical benefit.

The most commonly reported side effects were vomiting, nausea, acute liver injury, fever, and an abnormally low platelet count in the blood. Patients may also be at risk for severe muscle inflammation.

Inflammation of heart muscle and elevations of troponin I have also been seen following the use of Elevidys in clinical trials, the FDA said.

Accelerated approval of Elevidys was granted to Sarepta Therapeutics.

More information: More Information