FDA expands approval for Duchenne muscular dystrophy gene therapy

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The U.S. Food and Drug Administration has expanded the approval of Sarepta Therapeutics' Elevidys (delandistrogene moxeparvovec-rokl), a gene therapy for the treatment of Duchenne muscular dystrophy (DMD)

in individuals ≥4 years with DMD with a confirmed mutation in the DMD gene.

Elevidys is a single-dose, intravenous recombinant gene therapy designed to produce Elevidys micro-dystrophin, a shortened protein (138 kDa, versus the 427 kDa dystrophin protein of normal muscle cells) that contains selected domains of the dystrophin protein present in normal muscle cells.

The FDA granted traditional approval for ambulatory individuals ≥4 years with DMD with a confirmed mutation in the DMD gene, as well as accelerated approval in nonambulatory individuals ≥4 years. The traditional approval was based on two double-blind, placebo-controlled studies and two open-label studies (a total of 218 male participants).

While the trial Elevidys failed to meet its primary end point of improvement in the North Star Ambulatory Assessment versus placebo, it did meet key secondary outcome measures (time to rise from the floor, 10-meter walk/run, time to ascend four steps, and creatine kinase levels).

The FDA determined that "increased levels in micro-dystrophin are reasonably likely to predict clinical benefit in the nonambulatory population," supporting the accelerated approval in nonambulatory individuals. The most commonly reported side effects with Elevidys included nausea and vomiting, acute liver injury, fever, and thrombocytopenia.

"Today's approval broadens the spectrum of patients with Duchenne muscular dystrophy eligible for this therapy, helping to address the ongoing, urgent treatment need for patients with this devastating and life-threatening disease," Peter Marks, M.D., Ph.D., from the FDA Center for Biologics Evaluation and Research, said in a statement.

More information: More Information