FDA panel votes against approval of eteplirsen for DMD

April 27 2016

(HealthDay)—The drug eteplirsen should not be approved for treatment

of Duchenne muscular dystrophy, a U.S. Food and Drug Administration advisory panel said Monday.

There are currently no drugs to treat the disease. Patients, parents, and doctors insist the drug prolongs the ability of boys with Duchenne muscular dystrophy to walk long after they would normally be in wheelchairs, *The New York Times* reported. However, the FDA's panel of outside experts said clinical trial data from drug maker Sarepta Therapeutics did not meet the agency's requirements for well controlled studies necessary for approval. The vote was 7-3, with three abstentions.

The study submitted by Sarepta included only 12 patients and did not have adequate placebo control, *The Times* reported. The panel members found conflict between patient testimony and FDA rules. "Based on all I heard, the drug definitely works, but the question was framed differently," said Bruce Ovbiagele, M.D., chairman of neurology at the Medical University of South Carolina in Charleston, who voted against approval, *The Times* reported.

The FDA's decision on whether to approve eteplirsen will be made by May 26. The agency does not have to follow the recommendations of its advisory panels.

More information: More Information