Vamorolone effective, safe for boys with Duchenne muscular dystrophy

September 1 2022

For boys with Duchenne muscular dystrophy (DMD), vamorolone, a

structurally unique dissociative steroidal anti-inflammatory drug, is effective and safe over a 24-week period, according to a study published online Aug. 29 in *JAMA Neurology*.

Michela Guglieri, M.D., from the Newcastle Hospitals NHS Foundation Trust and Newcastle University in the United Kingdom, and colleagues examined whether vamorolone is able to retain efficacy while reducing safety concerns in DMD in a randomized, double-blind, multicenter study including boys aged 4 to younger than 7 years. The study included four treatment groups: placebo; prednisone 0.75 mg/kg/day; vamorolone 2 mg/kg/day; and vamorolone 6 mg/kg/day. One hundred twenty-one boys were randomly assigned to the treatment groups; 114 completed the 24-week treatment period.

The researchers found that the primary end point for change from baseline to week 24 time to stand velocity for vamorolone 6 mg/kg/day was met. The first four sequential secondary end points were also met: time to stand velocity, vamorolone, 2 mg/kg/day versus placebo; six-minute walk test, vamorolone, 6 and 2 mg/kg/day versus placebo; and time to run/walk 10 m velocity, vamorolone, 6 mg/kg per day versus placebo. In prednisone-treated, but not in vamorolone-treated participants, there were decreases in height percentile and bone turnover markers. Increased adrenal insufficiency was seen for all three treatment groups.

"The proven efficacy over a broad dose range (2 to 6 mg/kg per day) may enable physicians to adjust dose based on clinical observations and patient preferences," the authors write.

Several authors disclosed ties to biopharmaceutical companies, including ReveraGen, the manufacturer of vamorolone.

More information: Abstract/Full Text