FDA approves oral Duvyzat for Duchenne muscular dystrophy

March 26 2024, by Lori Solomon

The U.S. Food and Drug Administration has approved Duvyzat (givinostat) as an oral medication for the treatment of Duchenne muscular dystrophy (DMD) in patients six years of age and older.

Duvyzat is a histone deacetylase inhibitor administered orally twice daily

with food. The approval was based on results of a Phase III trial. All participants continued to receive a standard-of-care steroid regimen; half of the patients were also randomly assigned to Duvyzat.

After 18 months, patients treated with Duvyzat showed statistically significantly less decline in the time it took to climb four stairs (mean change in time to climb four stairs was 1.25 seconds with Duvyzat versus 3.03 seconds for placebo). During the 18 months, those treated with Duvyzat also saw less worsening in motor function as determined by the North Star Ambulatory Assessment.

The most common side effects of Duvyzat were diarrhea, abdominal pain, a decrease in platelets, nausea/vomiting, an increase in triglycerides, and fever. Prescribing information includes warnings that health care providers should evaluate the patient's platelet counts and triglycerides before prescribing.

"This approval provides another treatment option to help reduce the burden of this progressive, devastating disease for individuals impacted by DMD regardless of genetic mutation," Emily Freilich, M.D., of the FDA Center for Drug Evaluation and Research, said in a statement.

More information: FDA News Release