FDA approves firdapse for rare autoimmune disorder

November 30 2018

(HealthDay)—Firdapse (amifampridine) tablets have been approved by

the U.S. Food and Drug Administration for adults with Lambert-Eaton myasthenic syndrome (LEMS).

The drug is the first agency-sanctioned treatment for the autoimmune disease that affects the connections between nerves and muscles. LEMS affects about three people per one million worldwide, the FDA said. The disorder most commonly occurs among people with cancer, such as small cell lung cancer.

The drug's safety and effectiveness were evaluated in clinical studies involving 64 adults. Using a 13-item physician-rated categorical scale assessing muscle weakness and a seven-point scale on which patients rated their overall impression of the effects of the study treatment on their physical well-being, researchers found that those treated with Firdapse benefited more than those who took a placebo, the agency said. The drug's most common side effects included paresthesia, upper respiratory tract infection, abdominal pain, nausea, diarrhea, headache, and elevated liver enzymes. Patients who have a hypersensitivity reaction such as rash, hives, itching, fever, swelling, or trouble breathing should immediately contact their health care provider.

"Patients with LEMS have significant weakness and fatigue that can often cause great difficulties with daily activities," Billy Dunn, M.D., director of the FDA's Division of Neurology Products, said in an agency news release. "There has been a long-standing need for a treatment for this rare disorder."

Firdapse is produced by Catalyst Pharmaceuticals, based in Coral Gables, Florida.

More information: More Information