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FDA approves first treatment developed by Catalyst Pharma for LEMS

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In people with LEMS, the body's own immune system attacks the neuromuscular junction (the connection between nerves and muscles) and disrupts the ability of nerve cells to send signals to muscle cells.

The U.S. Food and Drug Administration has approved Firdapse (amifampridine) tablets for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults. LEMS is a rare autoimmune disorder that affects the connection between nerves and muscles and causes weakness and other symptoms in affected patients. This is the first FDA approval of a treatment for LEMS.

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

In people with LEMS, the body's own immune system attacks the neuromuscular junction (the connection between nerves and muscles) and disrupts the ability of nerve cells to send signals to muscle cells. LEMS may be associated with other autoimmune diseases, but more commonly occurs in patients with cancer such as small cell lung cancer, where its onset precedes or coincides with the diagnosis of cancer. The prevalence of LEMS is estimated to be three per million individuals worldwide.

The efficacy of Firdapse was studied in two clinical trials that together included 64 adult patients who received Firdapse or placebo. The studies measured the Quantitative Myasthenia Gravis score (a 13-item physician-rated categorical scale assessing muscle weakness) and the Subject Global Impression (a seven-point scale on which patients rated their overall impression of the effects of the study treatment on their physical well-being). For both measures, the patients receiving Firdapse experienced a greater benefit than those on placebo.

The most common side effects experienced by patients in the clinical trials were burning or prickling sensation (paresthesia), upper respiratory tract infection, abdominal pain, nausea, diarrhea, headache, elevated liver enzymes, back pain, hypertension and muscle spasms. Seizures have been observed in patients without a history of seizures. Patients should

inform their health care provider immediately if they have signs of hypersensitivity reactions such as rash, hives, itching, fever, swelling or trouble breathing.

The FDA granted this application Priority Review and Breakthrough Therapy designations. Firdapse also received Orphan Drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The FDA granted the approval of Firdapse to Catalyst Pharmaceuticals, Inc.