Generic Name:
Amifampridine
Trade Name:
Ruzurgi
Company:
Jacobus Pharmaceutical Co.
Notes:

On May 6, 2019, FDA approved amifampridine tablets for treatment of Lambert-Eaton myasthenic syndrome (LEMS) in patients aged 6 years to younger than 17 years. This is the first FDA approval of a treatment specifically for pediatric patients with LEMS. The only other treatment approved for LEMS is approved for use in adults.

LEMS is a rare autoimmune disorder in which the body?s immune system attacks the neuromuscular junction 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.

LEMS can occur at any age. The prevalence of LEMS specifically in pediatric patients is not known, but the overall prevalence of LEMS is estimated to be three per million individuals worldwide.

Use of amifampridine in patients aged 6 years to younger than 17 years is supported by evidence from adequate and well-controlled studies of the drug in adults with LEMS, pharmacokinetic data in adult patients, pharmacokinetic modeling and simulation to identify the dosing regimen in pediatric patients, and safety data from pediatric patients in that age group.

Effectiveness for treatment of LEMS was established by a randomized, double-blind, placebo-controlled withdrawal study of 32 adult patients in which patients were taking amifampridine for at least 3 months before entering the study. The study compared patients continuing on amifampridine to patients switched to placebo. Effectiveness was measured by the degree of change in a test that assessed the time it took the patient to rise from a chair, walk 3 meters, and return to the chair for three consecutive laps without pause. The patients who continued on amifampridine experienced less impairment than those on placebo.

Effectiveness was also measured with a self-assessment scale for LEMS-related weakness that evaluated the feeling of weakening or strengthening. The scores indicated greater perceived weakening in the patients switched to placebo.

The most common adverse effects experienced by pediatric and adult patients taking amifampridine were burning or prickling sensation, abdominal pain, indigestion, dizziness, and nausea. Adverse effects reported in pediatric patients were similar to those seen in adult patients. Seizures have been observed in patients without a history of seizures. Patients should inform their health professional immediately if they have signs of hypersensitivity reactions such as rash, hives, itching, fever, swelling, or trouble breathing.

Medication Monitor Categories:
New Drug Approvals