FDA approves ruzurgi for children with rare autoimmune disorder

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(HealthDay)—Ruzurgi (amifampridine) tablets are now approved to

treat Lambert-Eaton myasthenic syndrome (LEMS) in children aged 6 to 17 years, the U.S. Food and Drug Administration announced yesterday.

Ruzurgi is the first treatment to be approved for children with LEMS. Approval of the drug in this age group was based on evidence from controlled studies and pharmacokinetic data in adults with LEMS, as well as pharmacokinetic modeling and simulation and safety data among children aged 6 to 17 years to determine the dosing regimen for children with LEMS.

In a randomized, double-blind, placebo-controlled withdrawal study, 32 adult patients who had been taking Ruzurgi for at least three months were randomly assigned to continue taking Ruzurgi or switch to placebo. Patients who continued Ruzurgi had less impairment on a test assessing the time it took for a patient to rise from a chair, walk 3 m, and return to the chair for three consecutive laps without stopping. Patients' scores on a self-assessment scale for LEMS-related weakness also revealed greater perceived weakness for patients who switched to placebo compared with those who continued with Ruzurgi.

Commonly reported side effects included paresthesia, abdominal pain, indigestion, dizziness, nausea, and seizures.

Approval was granted to Jacobus Pharmaceutical Company Inc.

More information: More Information