Information for Patients and Caregivers

The Amifampridine Phosphate Expanded Access Program (EAP)

An investigational treatment may be available at no cost for patients with the neuromuscular disorders Lambert-Eaton Myasthenic Syndrome (LEMS) or certain types of Congenital Myasthenic Syndromes (CMS)

What is LEMS?

LEMS is a rare, autoimmune disorder that causes muscle weakness and affects muscle function.[1-5] In LEMS, your body attacks nerve endings that regulate a neurotransmitter controlling muscle contractions.[2,4] About 50%-60% of LEMS patients have an underlying cancer–most commonly small cell lung cancer.[2,3]

What is CMS?

Congenital Myasthenic Syndromes (CMS), are a group of rare, inherited diseases that primarily cause muscle weakness. CMS is caused by problems with various genes that affect communication between nerve and muscle cells.[6]

What is amifampridine phosphate?

Amifampridine phosphate is an investigational drug that has shown positive results for the treatment for LEMS in a recent U.S. clinical study. No serious side effects were reported. The most common side effects were: digital and oral paresthesias (e.g., tingling of the fingers, toes, or area around the mouth); nausea; and headache.

Amifampridine phosphate may be available at no cost for patients diagnosed with LEMS or certain types of CMS through the EAP.

Amifampridine phosphate has been approved and marketed for more than 5 years in the European Union for the treatment of LEMS. The FDA has not yet approved amifampridine phosphate for the symptomatic treatment of either LEMS or CMS.

What is an EAP?

Expanded Access Programs are intended to give patients access to investigational medications for serious diseases or conditions when there is no comparable or satisfactory treatment available. Participating physicians are able to obtain and provide the medication to their eligible U.S. patients while the medication is still under development.

What is the amifampridine phosphate EAP?

The EAP is designed to provide access to this investigational treatment for patients diagnosed with LEMS or certain types of CMS. At this time, amifampridine phosphate is only available through the EAP when a patient's treating physician feels this treatment can help improve their condition.

References: 1. Sanders DB. Lambert-Eaton myasthenic syndrome: diagnosis and treatment. Ann N Y Acad Sci. 2003;998:500-558. 2. Titulaer MJ, Lang B, Verschuuren JJ. Lambert-Eaton myasthenic syndrome: from clinical characteristics to therapeutic strategies. Lancet Neurol. 2011;10(12):1098-1107. 3. Titulaer MJ, Maddison P, Sont JK, et al. Clinical Dutch-English Lambert-Eaton Myasthenic Syndrome (LEMS) Tumor Association Prediction score accurately predicts small-cell lung cancer in the LEMS. J Clin Oncol. 2011;29(7): 902-908. 4. Oh SJ, Sieb JP. Update on amifampridine as a drug of choice in Lambert-Eaton myathenic syndrome. US Neurol. 2014;10(2):83-89. 5. Oh SJ, Kurokawa K, Claussen GC, Ryan HF Jr. Electrophysiological diagnostic criteria of Lambert-Eaton myasthenic syndrome. Muscle Nerve. 2005;32(4):515-520. 6. Engel AG, Shen XM, Selcen D, Sine SM. Congenital myasthenic syndromes: pathogenesis, diagnosis, and treatment. Lancet Neurol. 2015;14(4):420-434.

Catalyst pharmaceuticals

CALL TOLL FREE 1-844-347-3277 or
visit www.muscleweakness-eap.com

Amifampridine phosphate is an investigational drug and not currently commercially available.