Information for Physicians

The Amifampridine Phosphate Expanded Access Program (EAP)

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

The EAP is designed to provide access to treatment for patients diagnosed with LEMS or certain types of CMS. This investigational medication is only available through the EAP under the direction and care of the treating physician. The FDA has not yet approved amifampridine phosphate for the treatment of either LEMS or CMS.

AMIFAMPRIDINE PHOSPHATE DETAILS AND EAP ELIGIBILITY CRITERIA

Amifampridine phosphate

- Demonstrated positive results in one Phase 3 clinical study for LEMS
- Granted FDA Orphan Drug designation for LEMS and CMS
- Designated as a "Breakthrough Therapy" by the FDA for its review of the product for LEMS as a potentially substantial improvement over existing therapies
- Novel phosphate salt formulation allows for storage at room temperature
- Amifampridine phosphate is produced utilizing Good Manufacturing Practices (GMP) which ensure product quality and consistency
- Approved and marketed for more than 5 years in the EU for the treatment of LEMS

The most common adverse events in a Phase 3 clinical trial for this investigational treatment with LEMS patients were oral and digital paresthesias (eg, tingling) nausea, and headache

Patient eligibility criteria

Inclusion Criteria

- LEMS patients must be ≥10 years of age
- CMS patients must be ≥2 years of age
- Must have a confirmed diagnosis of LEMS or certain types of CMS

Exclusion Criteria

- History of epilepsy
- Known active brain metastases
- QTcB > 450 ms (males) or 470 ms (females)
- Hepatic impairment
    - o AST, ALT, or total bilirubin > 1.5 x upper limit of normal
- Creatinine clearance <30 mL/min

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.