MGFA Study Web Content:

Title of Study here

Study Purpose: To determine the efficacy and safety of amifampridine phosphate in improving the activities of daily living for patients with antibody positive MuSK myasthenia gravis.

Recruitment Information:

Study Type: Interventional

Status: Not yet recruiting

Target Patient: Myasthenia Gravis MuSK Antibody Positive

Eligible Ages: 18 Years and older (Adult, Senior)

Link to appropriate websites
https://clinicaltrials.gov/ct2/show/study/NCT03304054

Inclusion and Exclusion Criteria:

Inclusion Criteria: 1. Willing and able to provide written informed consent after the nature of the study has been explained and before the start of any research-related procedures. 2. Male or female ≥18 years of age. 3. Positive serologic test for anti-MuSK antibodies or anti-AChR antibodies as confirmed at Screening or by previous antibody test, with report available. 4. Confirmatory EMG or EMG report. 5. Myasthenia Gravis Foundation of America (MGFA) Class II to IV at Screening. 6. MG-ADL score of ≥6 at Screening, with more than 50% of this score attributed to non-ocular items. 7. Patients receiving steroids or pyridostigmine should not have any modification of drug regimen during the month before Screening. 8. Female patients of childbearing potential must have a negative pregnancy test (serum human chorionic gonadotropin [HCG] at screening); and must practice an effective, reliable contraceptive regimen during the study and for up to 30 days following discontinuation of treatment. 9. Ability to participate in the study based on overall health of the patient and

disease prognosis, as applicable, in the opinion of the Investigator; and able to comply with all requirements of the protocol, including completion of study questionnaires.

Exclusion Criteria: 1. Epilepsy and currently on medication. 2. Concomitant use of medicinal products with a known potential to cause QTc prolongation. 3. Patients with long QT syndromes. 4. History of thymectomy within 12 months before Screening. 5. An electrocardiogram (ECG) within 6 months before starting treatment that shows clinically significant abnormalities, in the opinion of the Investigator. 6. Breastfeeding or pregnant at Screening or planning to become pregnant at any time during the study. 7. Patients receiving immunomodulatory treatment (e.g. plasma exchange [PE], therapeutic plasma exchange [TPE], intravenous immunoglobulin G [IVIG]) should not have any treatment in the previous 4 weeks prior to Randomization or at any time during the study. 8. Use of rituximab or other similar biologic medications for immunomodulation within 6 months prior to Screening. 9. Treatment with an investigational drug (other than amifampridine) or device within 30 days before Screening or while participating in this study. 10. Any medical condition that, in the opinion of the Investigator, might interfere with the patient's participation in the study, poses an added risk for the patient, or confound the assessment of the patient. 11. History of drug allergy to any pyridine-containing substances or any amifampridine excipient(s).

<u>Study Information:</u>

Sponsor: Catalyst Pharmaceuticals, Inc.

Principal Investigator: Renato Mantegazza, MD

Study Coordinator: US contacts: Gary Ingenito, MD, PhD 305-420-3200, gingenito@catalystpharma.com or Adriana Manari 305-420-3200, amanari@catalystpharma.com

Type of Study: Interventional

Study Duration: 38 days excluding the screening period

Single Center/Multi-center: Multi-center

Travel Funds Available: ☒ Y ☐ N

Find A Center Near You:

List Sites Here

Site: Italy – various/ Carlo Besta Neurologic Institute

PI: Renato Mantegazza, MD

Coordinator: TBD

Site: US - Ohio State University

PI: Miriam Freimer, MD

Coordinator: Paige Matisak 614-685-5815 paige.matisak@osumc.edu

Site: Other US - TBD