Advancing treatment in MuSK-positive myasthenia gravis (MuSK-MG)

THE MuSK Trial—A Pivotal Trial For MuSK-MG Is Now Enrolling

Make a difference—help by referring eligible patients today!

Objective:

To evaluate the efficacy and safety of amifampridine phosphate in improving the activities of daily living for patients with MuSK antibody-positive MG.

Study Criteria:

Only adult patients with a positive test for anti-MuSK antibodies or anti-AChR antibodies can participate. In addition, patients must have a MG-ADL score of ≥6 (>50% non-ocular), a MGFA class II to class IV; and a confirmatory EMG or an EMG report upon screening.

Women of childbearing age cannot be pregnant and must remain on effective contraceptives throughout the study period and for 30 days after study completion.

Study Description:

THE MuSK Trial is a randomized, double-blind, placebo-controlled, parallel-group study. The study will include male and female participants (approximately 60 MuSK-MG and 10 AChR-MG patients). The intervention drug, amifampridine phosphate, will be orally administered 3 to 4 times/day, with a maximum dosage of 80 mg/day and not more than 20 mg per dose. Patients may continue on long-term pyridostigmine and/or corticosteroid therapy but neither of these therapies may be initiated, nor may dosage adjustments be made, within 30 days prior to patient screening.

Excluding the screening period (up to 14 days), patients will be randomized to receive either the investigational drug or placebo for approximately 38 days. The trial will be conducted at multiple sites across the United States and a limited number of sites in Italy. Close monitoring and 24-hour medical oversight will ensure safety. No invasive testing will be required and some of the study visits can be combined.

During the run-in period, the starting dosage of amifampridine phosphate will be 10 or 15 mg/day, with up-titration intervals every 3 to 4 days. After a maximum, tolerable dosing regimen has been established, the patient must remain stable at that dose for at least 7 days.

The run-in period will last approximately 3 weeks but may be extended if an optimal dose has not been achieved. At the end of the run-in period, **patients with ≥2-point improvement in MG-ADL score from baseline** will be eligible to enter the randomized study.

Open-label extension study:

This will be available to all patients who have completed **THE MuSK Trial** and signed an informed consent form. Patients who demonstrated benefit after completing the dose titration period but failed to meet the randomization criteria on Day 0 **may also be eligible for the extension study**. The evaluations for **THE MuSK Trial** may serve as baseline for the extension study, if available.

The optimal dosing regimen for amifampridine phosphate, from the end of the study or from the end of the run-in period, will be the dosage initially used for each patient in the extension study. Clinic visits for safety assessment and for evaluation of MG-ADL will be made at Months 3, 6, 9, 12, 15, and 21. Additional visits may occur at the discretion of the investigator.

Covered expenses:

Once eligibility has been established, the study will cover the cost of the investigational drug and all study-related activities. If necessary, travel accommodations, meals, and other expenses for both the patient and a support person may also be covered.

AChR=acetylcholine receptor; EMG=electromyography; MG=myasthenia gravis; ADL=activities of daily living; MGFA=Myasthenia Gravis Foundation of America; MuSK=muscle-specific kinase.

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

THE MuSK Trial

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Trial Summary

Medical condition under investigation: MuSK antibody-positive myasthenia gravis (MG); the purpose of the clinical trial is to determine the degree of MuSK-MG symptom control with amifampridine phosphate treatment.

Eligibility criteria: Adult patients with a positive anti-MuSK antibody test or a positive anti-AChR antibody test; if not previously tested, patients will be tested at the study site, at no charge.

Participant benefits: Once eligibility has been established, the study will cover the cost of the investigational drug and all study-related activities. If needed, travel accommodations, meals, and other expenses for both the patient and a support person may be covered, as well as access to expert medical care for the duration of the study, and access to long-term treatment for eligible patients in the open-label extension study.

Time commitment: THE MuSK Trial will be continuous for at least 38 days; the initial screening may take up to an additional 14 days. Note that some of the study visits can be combined.

Open-label extension study: Clinic visits for safety assessment and for evaluation of MG-ADL will be made at Months 3, 6, 9, 12, 15, and 21. Additional visits may occur at the discretion of the investigator

Study locations: Multiple sites across the United States and a limited number of sites in Italy.

NOT FOR PATIENT DISTRIBUTION

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To learn more about the trial, go to: www.clinicaltrials.gov/ct2/show/NCT03304054

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

This study is being sponsored by Catalyst Pharmaceuticals.