



CMS-001 Clinical Study

A clinical study for patients with Congenital Myasthenic Syndromes (CMS)

You may be eligible to participate in a new research study evaluating an investigational medicine for the treatment of CMS.

WHY a Study? Catalyst Pharmaceuticals and your doctor are trying to learn if the oral investigational medicine - amifampridine phosphate - can be safe and effective for the treatment of people with CMS. Much other testing has already occurred with this medicine, however in the US, a company must provide specific evidence from these type of studies to gain FDA approval.

Every study follows an extensive and carefully monitored process focused on the safety and privacy of the people who participate.

CMS is **rare** so every person that qualifies for the research study has an opportunity for treatment and the collective experience from all those who participate in the study will be reviewed to see if it can help to get the drug approved.

WHAT do You Need to Know?

- You may be eligible to participate if you are:
 - Ages 2 and older
 - Body weight ≥ 10 kg
 - Diagnosed with CMS involving certain genetically-confirmed defects
- Study Testing:
 - *Genetic testing will be provided to participants whose CMS has not been genetically-confirmed*
 - Other testing will include heart rhythm (ECG), muscle testing (EMG), questionnaires, and other testing which the study physician will describe
- Time Commitment:
 - Participation in the study is for 63 days, however, there are only certain times you would need to come to the clinic

After study completion, participants will be eligible to continue the medication by enrolling in an Expanded Access Program.

WHAT will You Need to Do?

1. Travel:

- You will need to travel to the study site.
- To assist you with all travel arrangements, the sponsor has contracted directly with the National Organization of Rare Disorders (NORD) to provide for and cover all travel-related costs for you and your caregiver/travel companion.

2. The Study:

You will be asked to give your approval in a signed document. This is called an informed consent. You may withdraw from the study at any time.

There are two stages for this research:

I. Screening: Your doctor will determine if you are eligible for the study.

- The study will consist of several clinical exams.
 - There will be an initial evaluation by the doctor at the site (i.e. screening visit). If you are eligible for the study it is possible you could return home the next day with medication to take for the next 4 weeks.

II. Treatment:

- It is a cross over study which means you will receive the active medicine and the placebo at different times; eventually ending up with the active medicine.
- You would need to return to the clinic the following week (day 7), at week 3 (day 21), and week 4 (day 29). **In total, there are 5 visits to the clinic.** The time at the clinic would be about a half day.
- At the day 1 evaluation the study will randomly select certain patients to get a placebo (non-study medication that looks like the study medication). This is considered a withdrawal from the medication and will be for 8 days.
- Another evaluation will occur at day 21 and then the patients who received placebo will be given study medication again.
- For the first 4 weeks, you will need to communicate over the phone with the doctor about how you are doing and the doctor will instruct you how to adjust the amount of medication you are receiving.
- After the 4 weeks, you would need to return to the clinic for follow up on your progress. If you are responding to treatment, then you would continue in the study (day 1).

3. Once the study is complete, all will have the opportunity to be enrolled in an **Expanded Access Program** which allows the physician to continue to provide the medication to the study participants.

If you are interested in the study, please contact EAP@catalystpharma.com or 1-844-347-3277 or any of the sites listed below:

Children's Healthcare of Atlanta
Atlanta, Georgia, 30342

Contact: Schauna Gillam 678-409-4612
schauna.gillam@choa.org

Boston Children's Hospital
Boston, Massachusetts, 02115

Contact: Grace Ordonez 617-919-7384
grace.ordonez@childrens.harvard.edu

Johns Hopkins Pediatric Neurology
Baltimore, Maryland, 21287

Contact: Agnes King Rennie, CNA,
MST 443-287-6294
aking2@jhmi.edu

Nationwide Children's Hospital
Columbus, Ohio 43205

Contact: Alana Mahley 614-355-2606
alana.mahley@nationwidechildrens.org

UCLA

Los Angeles, CA, 90095

Contact: Angela Ho, 310.825-3264
ALHo@mednet.ucla.edu

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