Catalyst pharmaceuticals

Neuromuscular Connections

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Thank You for Connecting

Welcome to Catalyst Neuromuscular Connections (CNMC) - our connection point to the community. This bulletin is designed to facilitate conversations between Catalyst and people living with neuromuscular conditions. CNMC will help you get to know our employees, learn about our patient support programs, understand what we're up to, and most importantly, provide you with a forum to pose questions, answer polls, and hear patient stories. We hope that you will find it interesting and informative.

Catalyst News

We've been busy connecting with patients, researchers, and physicians at neuromuscular and rare disease conferences across the country.

Myasthenia Gravis Foundation of America (MGFA) National Conference (April, 2018)
Members of the Catalyst medical affairs team introduced a MuSK-MG clinical study that is currently enrolling patients at the MGFA annual conference, a 3-day event dedicated to providing patients and families with the latest disease information and research.

Global Genes RARE Patient Advocacy Summit (October, 2018)
The RARE Patient Advocacy Summit offers one of the largest educational events for rare disease patients and advocates. Catalyst was on hand to connect with the community and several LEMS patients had the opportunity to gather for a meetup made possible in part by a grant from Catalyst.

American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) Annual Meeting (October, 2018)
AANEM unites professionals dedicated to the advancement of treatment for neuromuscular disorders. During the AANEM annual meeting, Catalyst sponsored the Myasthenia Gravis Foundation of America (MGFA) Scientific Conference, which provided an engaging venue for investigators to share pre-publication data and obtain vital feedback from other scientists and medical professionals.

American Neurological Association (ANA2018) Congress of Neurological Surgeons (CNS) (October, 2018)
To help educate physicians on how to recognize LEMS and CMS, Catalyst hosted a "Medical Mystery" presentation at each of these conferences. This fun and interactive format requires the audience to identify the disease based on the information presented in simulated visit between a patient and physician.

National Organization for Rare Disorders (NORD) 2018 Rare Diseases and Orphan Products Breakthrough Summit (October, 2018)
We met with leaders of NORD and other organizations to learn how to better advocate with legislators in the hopes of ensuring that patients with rare diseases have the abilities to access and pay for the medicines that help them manage their unique conditions.

New Drug Application (NDA) Accepted!

Catalyst announced in May that the U.S. Food and Drug Administration (FDA) had accepted the New Drug Application (NDA) for amifampridine phosphate as a potential treatment for Lambert-Eaton myasthenic syndrome (LEMS). An NDA is a formal proposal from a pharmaceutical company to the FDA requesting approval of a new drug in the United States. The FDA usually takes between 5-10 months to review an NDA before making a decision to approve the medicine for marketing.

The FDA has granted priority review status to the amifampridine phosphate NDA. Regulators take this step when they believe that a faster review and approval of a medicine will provide some benefit to patients.

The FDA has issued an action date (the day they have to make a decision about approving a medicine) of Nov. 28, 2018. If approved, amifampridine phosphate will become the first medicine approved to treat LEMS in over 30 years and will be available by prescription to all patients with LEMS. Catalyst is also conducting clinical studies in other neuromuscular diseases including congenital myasthenic syndromes (CMS) and muscle specific tyrosine kinase autoantibody myasthenia gravis (MuSK-MG).

Find more Catalyst News updates here.

What is LEMS?

- LEMS is a rare and severe autoimmune disease that causes progressive, general muscle weakness, especially in the legs.
- People with LEMS find it difficult to do simple daily activities like walk, stand up from a chair, climb steps, or get out of a car or bed.

- LEMS is a presynaptic disease because it affects nerve cells just before the synapse, which is the space separating the nerve ending from the muscle cell.
- While LEMS can appear at any age, it usually occurs in people who are 40 years or older. It is important that people with LEMS get screened for cancer because LEMS can be associated with cancer. Experts recommend screening shortly after diagnosis and again at three to six months and then every six months for the next two years.

Meet Our Team

Gary Ingenito, MD, PhD, Chief Medical Officer

Gary Ingenito, MD, PhD, our Chief Medical Officer, oversees our clinical studies and drug development programs. He led the drug development program, including clinical trials and the New Drug Application (NDA) for amifampridine phosphate.

Dr. Ingenito is a neurologist with a PhD in immunology. He chose neurology because, the neurosciences and neurophysiology "spoke" to him during medical school, so the field of neurology "just made sense." Following his residency, he accepted his first role in drug development because "it seemed like a great way to touch more patients' lives than I could in clinical practice." He has been in one facet of drug development or another ever since. He believes that one of the best parts about his job is that he never stops learning.

When asked what he loves about Catalyst, he says, "It's like a family here. We really care about people with rare diseases and understand what a long and difficult road they often have to getting a correct diagnosis. Catalyst appreciates every one of those patients and is dedicated to ensuring that they have access to medications."

"I know the work I do every day, the decisions I make about clinical studies and drug development can eventually have tremendous impact on the day-to-day lives of people living with neuromuscular disease."

Community Spotlight

Catalyst Patient Engagement Charter

Learning from the Patient Community

Catalyst is committed to improving the lives of people living with rare and debilitating neuromuscular diseases. How do we go about this? We start with the patient community because we know that understanding their true experiences and needs will help us make the greatest impact. The perspective we gain from the community guides our decisions and shapes how we move forward.

The guiding principles for our patient engagement are as follows:

- We recognize and respect the autonomy of our advocacy partners and seek to reinforce their independence and integrity. We will not place our interests above theirs.
- We will not request or expect any patient organization to promote a Catalyst Pharmaceuticals product.
- We will be open and transparent about the objectives and scope of any collaboration with patient organizations.
- We will respect and guard the privacy of all personal information and data we receive from patients and patient organizations. We will only release information if given consent.
- We strongly encourage patient organizations to pursue and establish multiple funding sources.
- We will acknowledge Catalyst's support and sponsorships of such patient organizations.

Connections Corner

Welcome to the Connections Corner, where we want to hear from you. If you have a question about LEMS or neuromuscular diseases, it is likely that others do too.

Send your answers to one or more of the questions below to CNMCeditor@catalystpharma.com so that we can share your valuable insights with other members of the community.

- What is the one thing you want to know but have not asked your doctor about LEMS?
- What is the one thing you wish someone would have told you about LEMS?
- What is the one thing you would tell someone recently diagnosed with LEMS?
- How long did it take to get your initial LEMS diagnosis?
- How did it make you feel to receive the diagnosis?

Community Q&A

Q: How long did it take to get your initial LEMS diagnosis?

A: "It took me 6 years to get a LEMS diagnosis. I was told my muscle weakness was due to my weight, pre-menopause, or just getting older. I kept pushing and finally was sent to a neurologist by my optometrist."

– Lisa, a woman living with LEMS

To learn more about Lisa and to see other patient stories, visit the Catalyst website at https://catalystpharma.com/lems-stories/

If you have comments or questions about anything in CNMC, write to us at CNMCeditor@catalystpharma.com and we will do our best to respond, either directly or in future issues.

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